

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

January 9, 2019

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SCIENCE.**  
ACORDA  
THERAPEUTICS

WE WILL FIND A WAY  
WE WILL MAKE THE  
COMMUNICATION COMMUNICATION  
COMMUNICAT  
INTEGRITY... WE'RE FULL O  
WE TELL IT LIKE IT IS  
WE DON'T SMOOTH OR COVER UP  
THE MESSAGE  
TEAMWORK UH, HUH  
THERAPIES OR BUST!!  
WE DON'T SMOOTH OR COVER UP  
THE MESSAGE  
WE MAKE BEANS COUNT  
BUT WE HAVE FUN

# Forward Looking Statement

This presentation includes forward-looking statements. All statements, other than statements of historical facts, regarding management's expectations, beliefs, goals, plans or prospects should be considered forward-looking. These statements are subject to risks and uncertainties that could cause actual results to differ materially, including: we may not be able to successfully market Inbrija or any other products under development; risks associated with complex, regulated manufacturing processes for pharmaceuticals, which could affect whether we have sufficient commercial supply of Inbrija to meet market demand; third party payers (including governmental agencies) may not reimburse for the use of Inbrija or our other products at acceptable rates or at all and may impose restrictive prior authorization requirements that limit or block prescriptions; competition for Inbrija, Ampyra and other products we may develop and market in the future, including increasing competition and accompanying loss of revenues in the U.S. from generic versions of Ampyra (dalfampridine) following our loss of patent exclusivity; the ability to realize the benefits anticipated from acquisitions, among other reasons because acquired development programs are generally subject to all the risks inherent in the drug development process and our knowledge of the risks specifically relevant to acquired programs generally improves over time; we may need to raise additional funds to finance our operations and may not be able to do so on acceptable terms; the risk of unfavorable results from future studies of Inbrija (levodopa inhalation powder) or from our other research and development programs, or any other acquired or in-licensed programs ; the occurrence of adverse safety events with our products; the outcome (by judgment or settlement) and costs of legal, administrative or regulatory proceedings, investigations or inspections, including, without limitation, collective, representative or class action litigation; failure to protect our intellectual property, to defend against the intellectual property claims of others or to obtain third party intellectual property licenses needed for the commercialization of our products; and failure to comply with regulatory requirements could result in adverse action by regulatory agencies.

These and other risks are described in greater detail in our filings with the Securities and Exchange Commission. We may not actually achieve the goals or plans described in our forward-looking statements, and investors should not place undue reliance on these statements. Forward-looking statements made in this press release are made only as of the date hereof, and we disclaim any intent or obligation to update any forward-looking statements as a result of developments occurring after the date of this presentation.

## OUR MISSION

Developing therapies  
to restore function and  
improve the lives of  
people with  
neurological disorders



# 2018 Highlights



## INBRIJA™ Approval

- For the intermittent treatment of OFF episodes in people with Parkinson's (PwP) taking carbidopa/levodopa



## Strong Financial Position

- Year end cash balance of ~\$445 million



# INBRIJA™ (levodopa inhalation powder)

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THERAPEUTICS

**Inbrija**<sup>™</sup>  
(levodopa inhalation powder)  
42 mg capsules

**INBRIJA is Approved for Intermittent Treatment of OFF Episodes in People with Parkinson's (PwP) Taking Carbidopa/Levodopa**



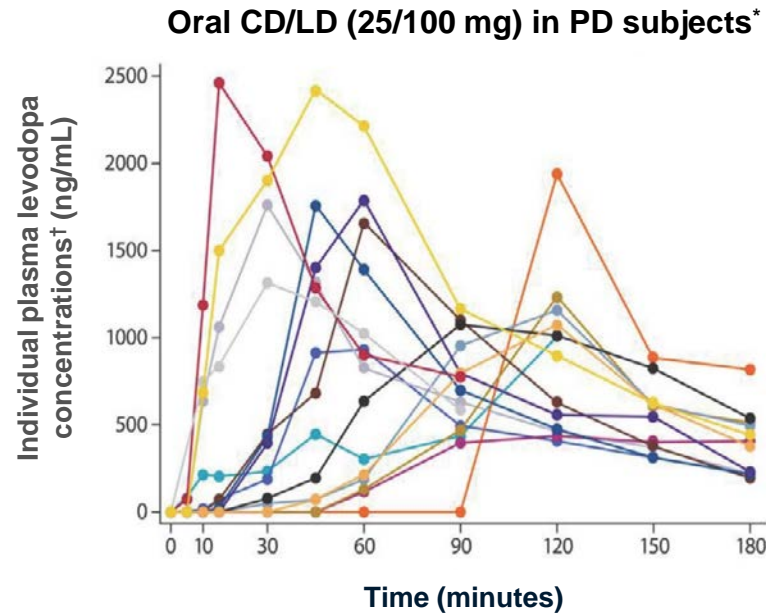
# Challenges of Oral Levodopa

## Parkinson's GI effects<sup>1</sup>



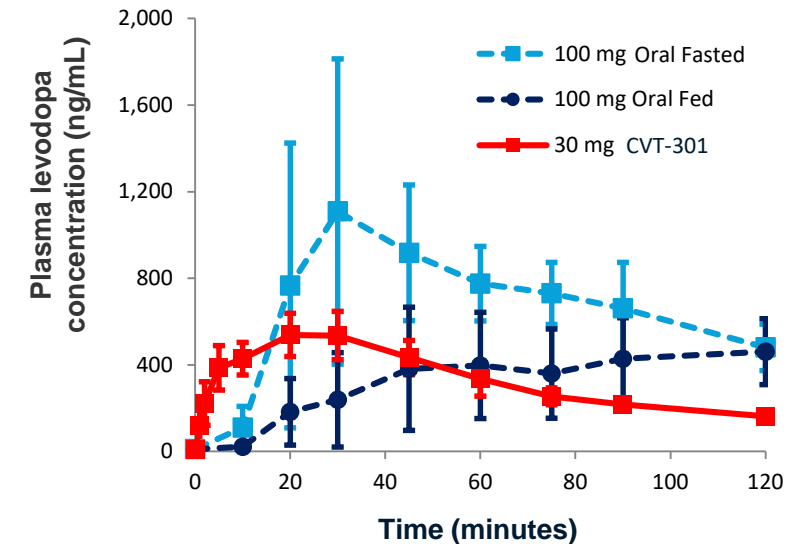
**Delay in Gastric Emptying:** Arrow points to a carbidopa tablet remaining intact in a patient's stomach about 1.5 hours after intake

## Variable Pharmacokinetics<sup>†,3,4</sup>



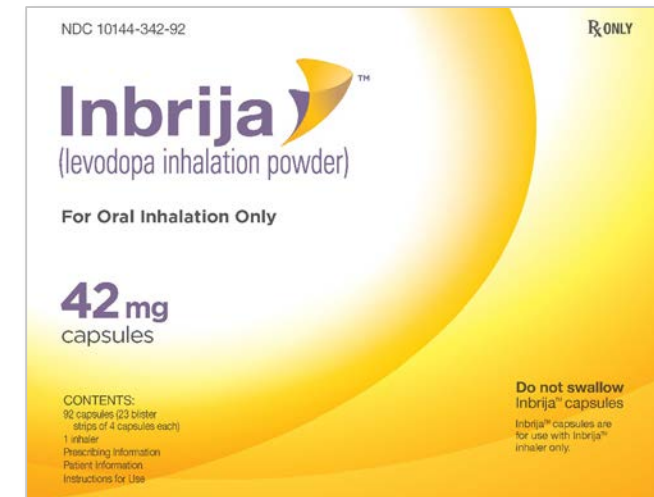
\*No food at least 1 hour before and 1 hour after CD/LD dose  
† Variable-adjusted

## CVT-301 Phase 1 trial in healthy volunteers



# INBRIJA Label

- On-demand use
- Onset as early as 10 minutes that reached statistical significance by 30 minutes
- Single dosage strength 84mg; no titration
- One year open-label safety data





# SPAN-PD Phase 3 Trial

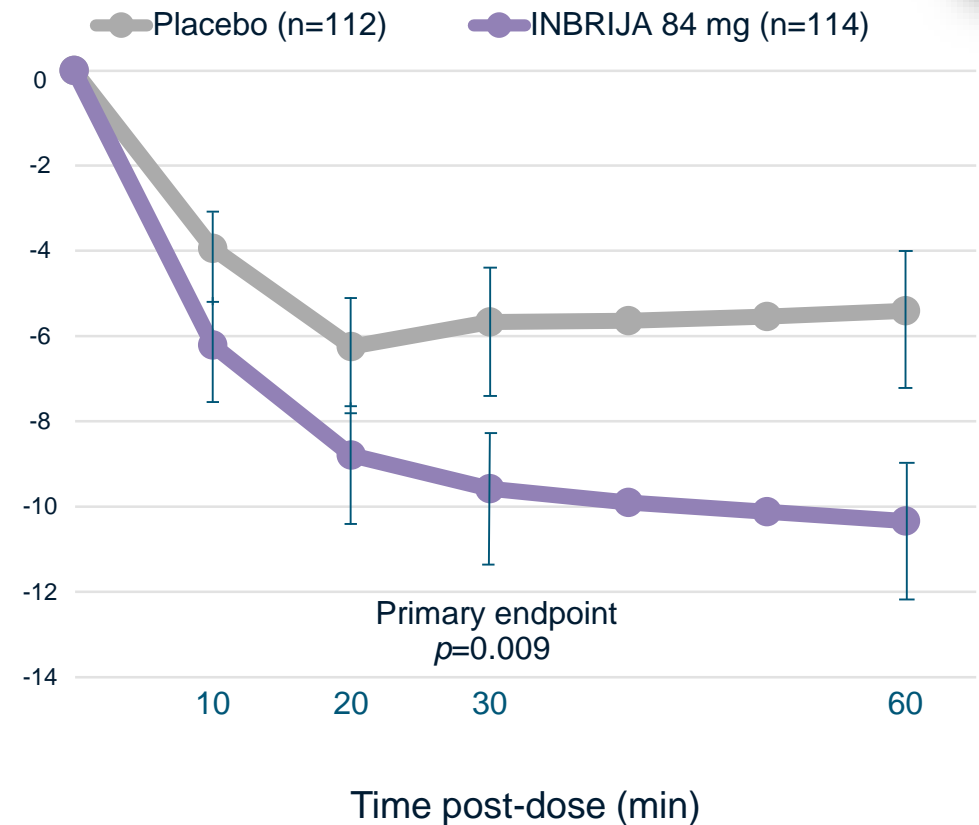
## UPDRS III score change from 0-60 minutes post-dose at week 12

- **Onset of action:**  
as early as 10 minutes post-dose
- **Primary endpoint:**  
significant improvement in motor function at 30 minutes post-dose ( $p=0.009$ )
- **Continuation of effect:**  
60 minutes post-dose

Worsening

Least squares mean ( $\pm$  SE)  
change from predose

Improvement



# SPAN-PD: Adverse Reactions Over 12 Weeks<sup>1,2</sup>

Adverse reactions occurring in  $\geq 5\%$  of INBRIJA-treated patients and more frequently than placebo

	INBRIJA 84 mg (n=114)	Placebo (n=112)
Cough	15%	2%
Upper respiratory tract infection	6%	3%
Nausea	5%	3%
Sputum discolored	5%	0%

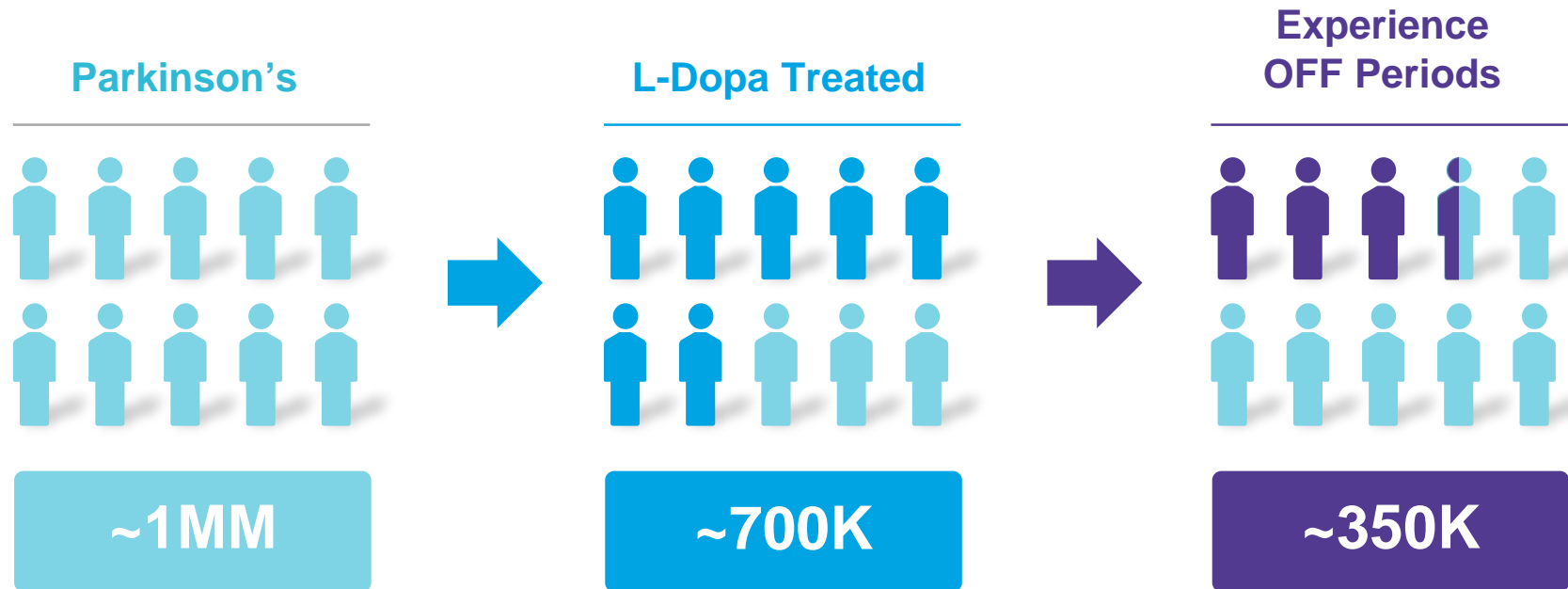
## Discontinuations due to adverse reactions:

- INBRIJA 84 mg group: 6 participants (5.3%)
- Placebo group: 3 participants (2.7%)

## Most common adverse reaction was cough

- Inhalation of INBRIJA can lead to coughing at the time of administration.
- For INBRIJA 84 mg, 11 of 17 participants reported cough as mild, and 6 reported as moderate in severity
  - 2 participants discontinued due to cough

# Significant Unmet Need

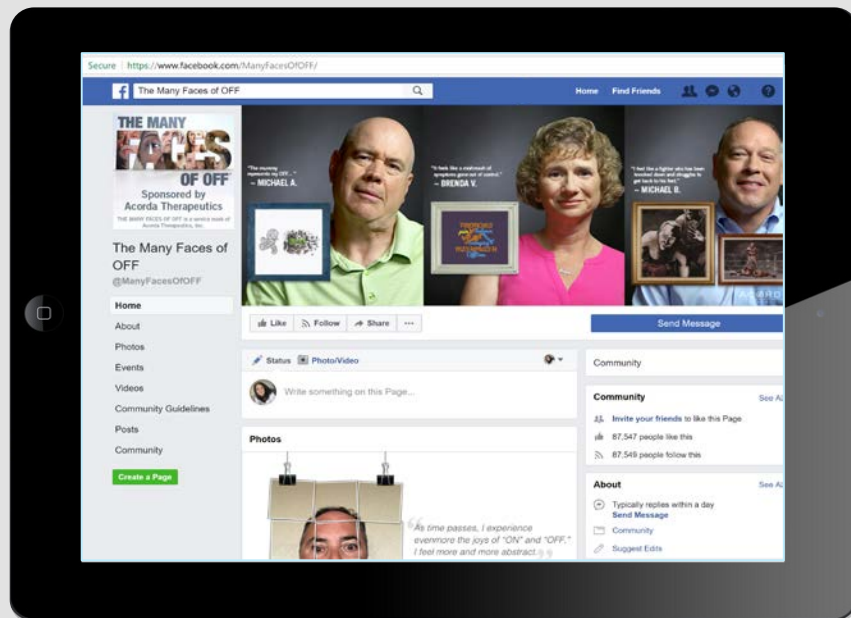


 = 100,000 people

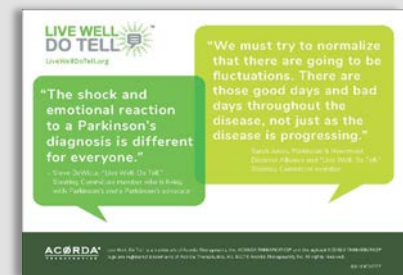
Source: National Parkinson's Foundation

# Multi-Channel Parkinson's Disease Awareness

## The Many Faces of OFF Facebook Page



## Live Well. Do Tell. Website





# Proven Neuro-Specialty Commercial Organization

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# INBRIJA Market Research

## Extensive Discussions / Surveys

**>600**



**PwPs  
Caregivers**

**>1,000**



**Neurologists  
MD Specialists  
HCPs**



## Consistent Themes

- ✓ Oral levodopa “gold standard”
- ✓ Significant unmet need
- ✓ Burden of OFF
- ✓ On-demand use for OFF
- ✓ Likelihood to prescribe and/or discuss

# INBRIJA Market Research



## Burden of OFF

Nearly **80%** of OFF periods are bothersome



## Unmet Need

**68%** find OFF periods at least somewhat difficult to manage



## Prescribing

**78%** likely to discuss INBRIJA with their doctor



PwP



HCP

**86%** agree OFF periods can be very bothersome to their PD patients

Characterized **64%** of their PD patients' OFF periods as moderate or severe

**73%** very or extremely likely to prescribe INBRIJA for their patients

# INBRIJA Launch Execution

- **Leading neuro-specialty commercial organization**
- **Strong relationships with managed care, HCPs and Parkinson's community**

## Field Team Deployment

- Product training and demonstration at Movement Disorder/key neurology centers



## INBRIJA in Channel

- Samples available
- Branded campaign goes live



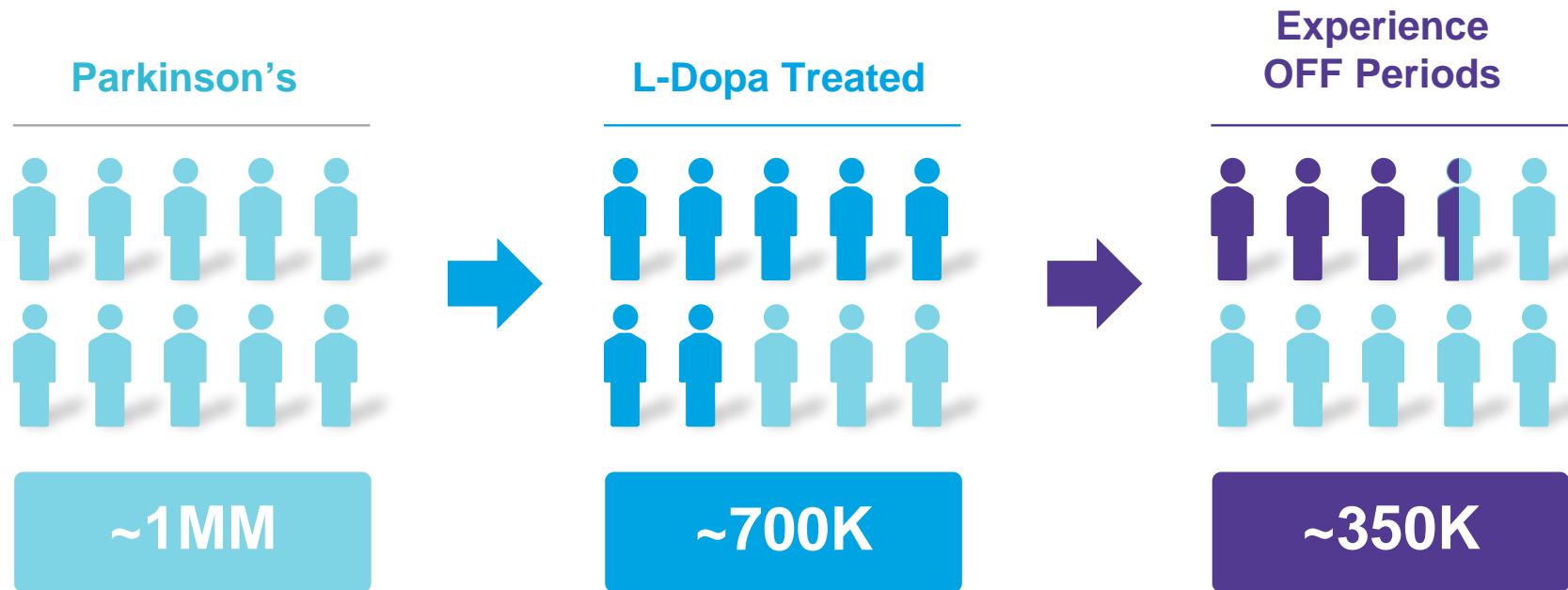
## Hub and Patient Services

- INBRIJA Start Kit
- Reimbursement assistance
- [www.INBRIJA.com](http://www.INBRIJA.com)



# INBRIJA U.S. Market Opportunity

## Projected Peak U.S. Sales >\$800 million



 = 100,000 people

Source: National Parkinson's Foundation



# Innovative ARCUS<sup>®</sup> Technology

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# ARCUS Platform



## Large Porous Particles for Pulmonary Drug Delivery

David A. Edwards,\* Justin Hanes, Giovanni Caponetti, Jeffrey Hrkach, Abdelaziz Ben-Jebria, Mary Lou Eskew, Jeffrey Mintzes, Daniel Deaver, Noah Lotan, Robert Langer\*

A new type of inhalation aerosol, characterized by particles of small mass density and large size, permitted the highly efficient delivery of inhaled therapeutics into the systemic circulation. Particles with mass densities less than 0.4 gram per cubic centimeter and mean diameters exceeding 5 micrometers were inspired deep into the lungs and escaped the lungs' natural clearance mechanisms until the inhaled particles delivered their ther-



SCIENCE TRANSLATIONAL MEDICINE | RESEARCH ARTICLE

## PARKINSON'S DISEASE

### Preclinical and clinical assessment of inhaled levodopa for OFF episodes in Parkinson's disease

Michael M. Lipp,<sup>1\*</sup> Richard Batycky,<sup>1</sup> Jerome Moore,<sup>2</sup> Mika Leinonen,<sup>3</sup> Martin I. Freed<sup>1†</sup>

Inhaled drugs offer advantages, such as rapid onset of action, but require formulations and delivery systems that reproducibly and conveniently administer the drug. CVT-301 is a powder formulation of levodopa delivered by a breath-actuated inhaler that has been developed for treating OFF episodes (motor fluctuations between doses of standard oral levodopa) in patients with Parkinson's disease (PD). We present preclinical, phase 1, and phase 2 results for CVT-301. In dogs insufflated with a levodopa powder, plasma levodopa peaked in all animals 2.5 min after administration; in contrast, in dogs dosed orally with levodopa plus carbidopa, plasma levodopa was not detected until 30 min after

Edwards, D et al. Large Porous Particles for Pulmonary Drug Delivery. Science 276, 1868-1871 (1997).

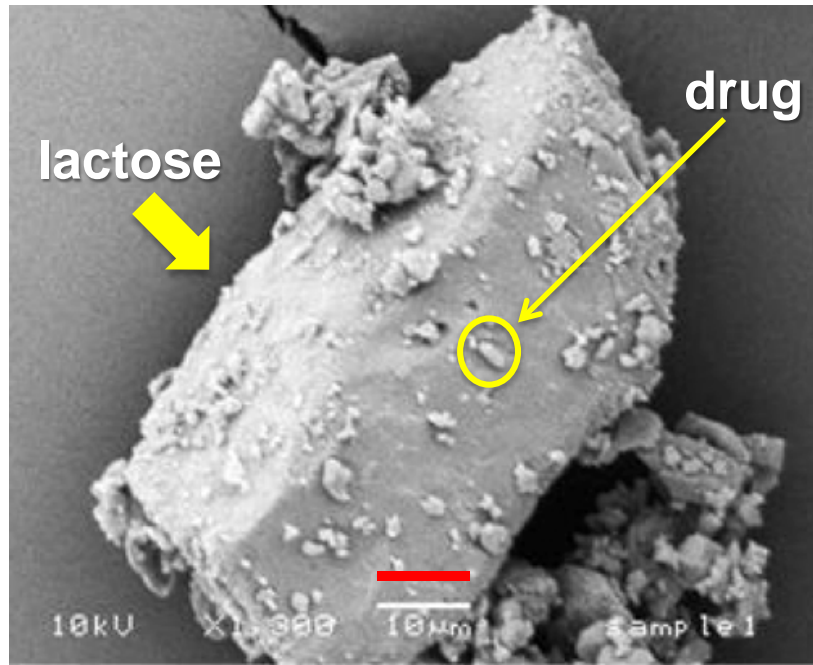
Lipp M, Batycky, R, Moore, J, Leinonen, M, Freed, M. Preclinical and clinical assessment of inhaled levodopa for OFF episodes in Parkinson's disease. Science

Translational Medicine 8, 360ra136, 1-10 (2016). 19

# Innovative ARCUS Technology

Small/dense drug particles – require use of lactose blends for dispersibility

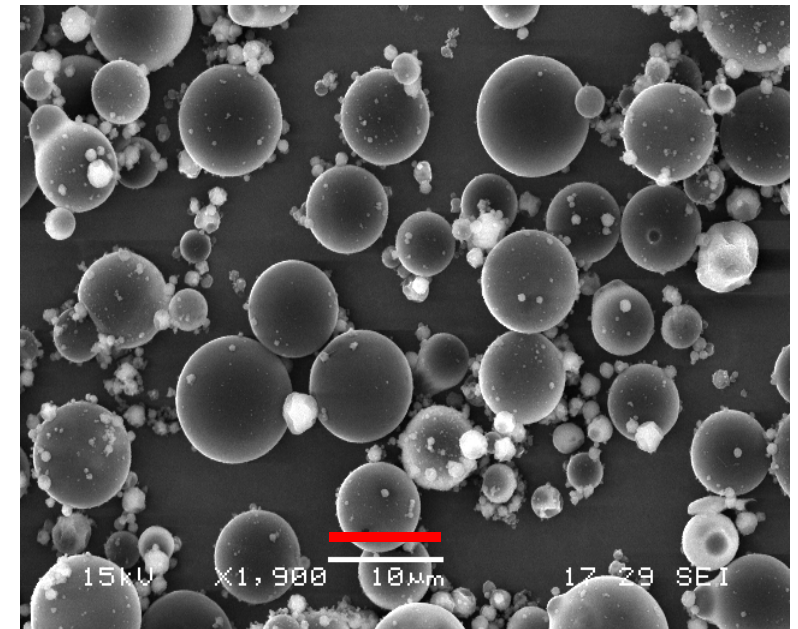
— = 10 microns



- Geometric size
- Dispersibility
- Density
- Cohesiveness

## ARCUS

— = 10 microns

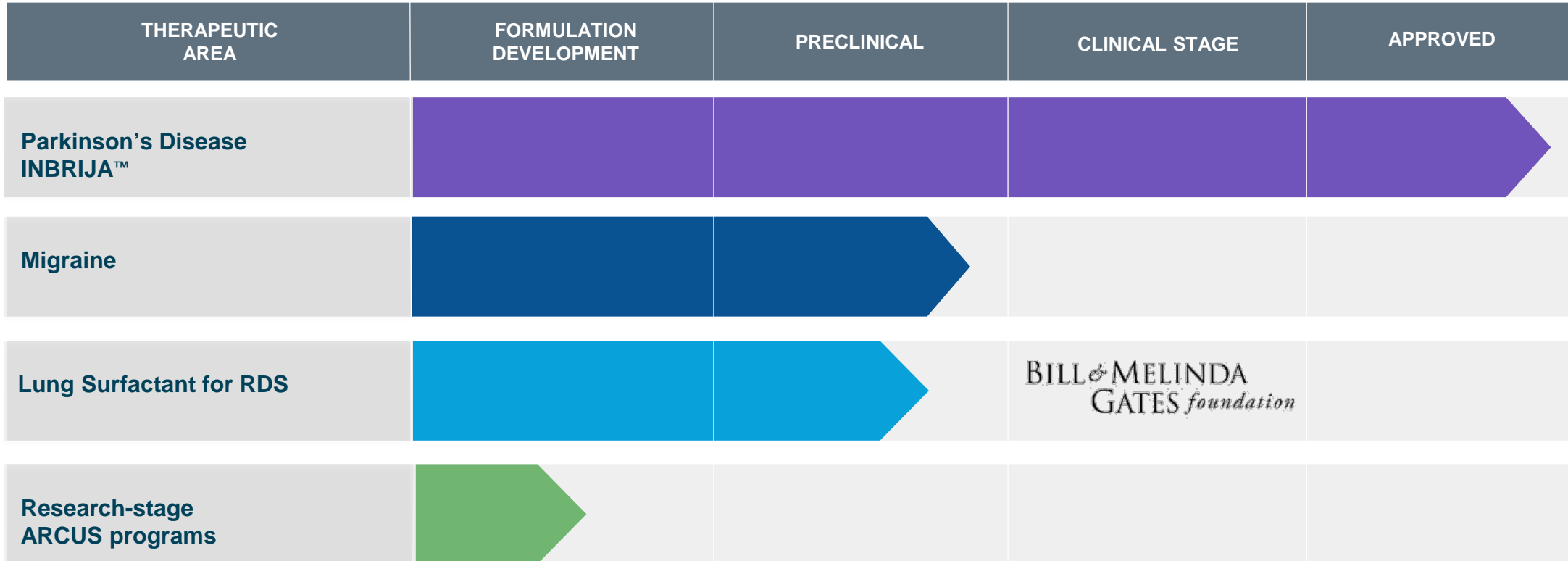


# ARCUS for Acute Migraine

- Proof of concept with zolmitriptan
  - Median  $T_{\max}$  was ~12 minutes for all doses vs 1.5 hours for oral tablet and 3 hours for nasal spray
- Formulation development progressing for 3 different migraine drugs
- Large market opportunity



# ARCUS Pipeline Opportunities





# 2019 Guidance and Priorities

# 2019 Expense Guidance



**R&D Expense**  
\$70 - \$80 million



**SG&A Expense**  
\$200 - \$210 million

2019 R&D and SG&A expense guidance are non-GAAP financial measures which exclude share-based compensation. Information about our use of non-GAAP financial measures, and a description of the excluded items, is available in our January 7, 2019 press release.



# 2019 Strategic Priorities

## INBRIJA

- U.S. commercial launch
- Obtain MAA approval
- Ex-U.S. partnering strategy

## ARCUS Platform

- Advance pipeline candidates

## Financial Management

- Maintain strong balance sheet

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