



| DISCOVER  
| DEVELOP  
| DELIVER

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**Novel Medicines for  
Life-Altering CNS  
Disorders**

**Q4 & FY 2015 Financial Results  
February 24, 2016**

# Agenda

## **Introduction**

- Paul Cox, Director, Investor Relations

## **Pipeline Update and Upcoming Milestones**

- Jeff Jonas, M.D., Chief Executive Officer

## **Financial Results**

- Kimi Iguchi, Chief Financial Officer

## **Q&A Session**

# Forward-Looking Statements

These slides and the accompanying oral presentation contain forward-looking statements, which may be identified by the use of words such as “may,” “might,” “will,” “should,” “expect,” “plan,” “anticipate,” “believe,” “estimate,” “project,” “intend,” “future,” “potential,” or “continue,” and other similar expressions. Forward-looking statements in this presentation include statements regarding: the potential safety, pharmacological effect and efficacy of SAGE’s product candidates; the expected development pathway for SAGE’s product candidates; anticipated development milestones and results, including expected timing; the anticipated impact of SAGE’s development model on future development results and on its ability to advance its pipeline; potential future indications for SAGE’s product candidates; other planned activities and business outlook; and SAGE’s expectations with respect to cash needs. These forward-looking statements are neither promises nor guarantees of future performance, and are subject to a variety of risks and uncertainties, many of which are beyond SAGE’s control, which could cause actual results to differ materially from those contemplated in these forward-looking statements, including the risk that:

- SAGE may not be able to successfully demonstrate the efficacy and safety of its product candidates at each stage of development;
- success in SAGE’s pre-clinical studies or in early stage clinical trials may not be repeated or observed in ongoing or future studies involving the same compound or other product candidates, and future pre-clinical and clinical results for SAGE’s product candidates may not support further development of the product candidate or regulatory approval;
- decisions or actions of regulatory agencies may affect the initiation, timing and progress of clinical trials, or SAGE’s ability to obtain marketing approval for its product candidates;
- we may experience slower than expected clinical site initiation or slower than expected identification and enrollment of evaluable patients in our clinical trials, or may encounter delays or problems in analyzing data or the need for additional analysis, data or patients.

- even if SAGE’s products are successfully developed and approved, the actual market for such products may be smaller than SAGE’s current estimates;
- SAGE may not be able to obtain and maintain adequate intellectual property protection or other forms of data and marketing exclusivity for its products, or to defend its patent portfolio against challenges from third parties;
- SAGE may face competition from others developing products for similar uses as those for which SAGE’s products are being developed;
- SAGE’s operating expenses may be higher than forecasted and SAGE may also face unexpected expenditures, in either case which may result in the need for additional funding to support its business activities earlier than anticipated;
- Funding to support operations may not be available, when needed, on reasonable terms or at all, or may result in significant dilution to existing shareholders;
- SAGE may not be able to establish and maintain key business relationships with third parties on whom SAGE is, or will need to be, dependent for development or manufacture of products or for future marketing, sales and distribution of products, if SAGE is successful in its development efforts;
- SAGE may encounter technical and other unexpected hurdles in the manufacture and development of its products.

For additional disclosure regarding these and other risks SAGE faces, see the disclosure contained in the “Risk Factors” section of SAGE’s Quarterly Report on Form 10-Q filed on November 6, 2015, and in SAGE’s other public filings with the Securities and Exchange Commission, available on the SEC’s website at <http://www.sec.gov>. Any forward-looking statement represent SAGE’s views only as of today, and should not be relied upon as representing its views as of any subsequent date. SAGE undertakes no obligation to update or revise the information contained in this presentation, whether as a result of new information, future events or circumstances or otherwise.

# Innovating Medicines for Life-Altering CNS Disorders

**Our mission is to make life better for patients with CNS disorders by**

| **DISCOVERING**

| **DEVELOPING**

| **DELIVERING**

**important new medicines for patients in need**

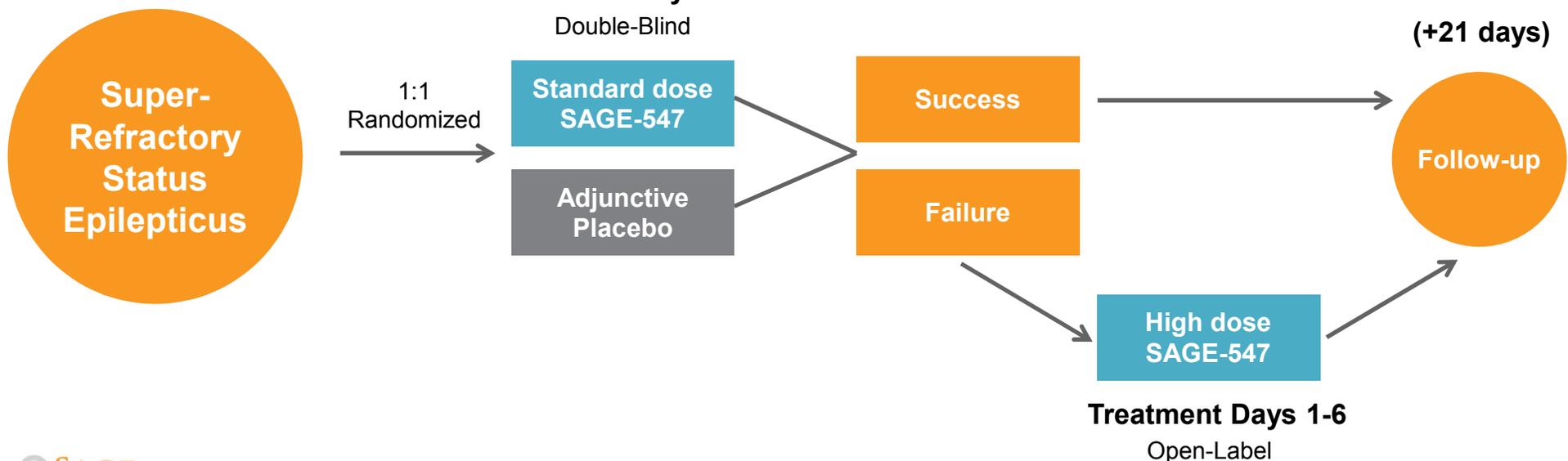


# Global Phase 3 STATUS Trial of SAGE-547 in SRSE

## STATUS TRIAL

**Top-line results expected  
2H 2016**

- Randomized, double-blind, placebo-controlled
- Expect up to 140 patients enrolled to get 126 evaluable patients
- Anticipate ~150 sites in U.S., Canada and Europe
- Non-responders eligible for open-label, SAGE-547 retreatment
- SPA agreement with FDA
- Primary Efficacy Endpoint: Continued resolution of SE for 24 hours following wean of all 3rd-line agents and SAGE-547/placebo



# Building a Wholly-Owned Multi-Product CNS Portfolio

Discovery

Preclinical

Phase 1

Phase 2

Phase 3

## GABA<sub>A</sub> Dysfunction-Related Disorders

Super-Refractory Status Epilepticus

SAGE-547

Severe Postpartum Depression

SAGE-547 Proof-of-Concept

Essential Tremor

SAGE-547 Proof-of-Concept

SAGE-217

Orphan Epilepsies

SAGE-217

Status Epilepticus

SAGE-689

GABA Indications

SAGE-105/324

## NMDA Dysfunction-Related Disorders

Smith-Lemli-Opitz Syndrome

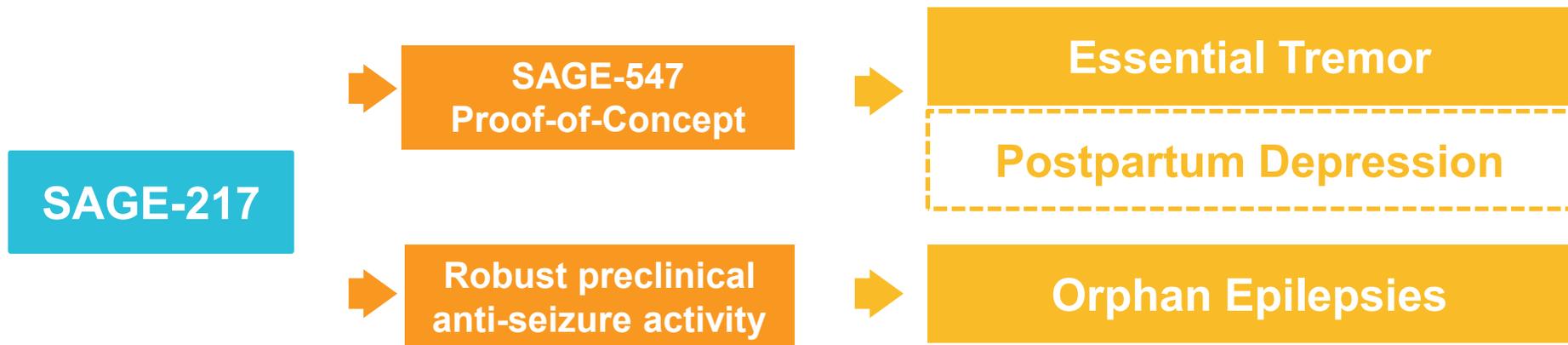
SAGE-718

Anti-NMDA Receptor Encephalitis

SAGE-718

NMDA Indications

# Robust Phase 2 Development Programs Planned

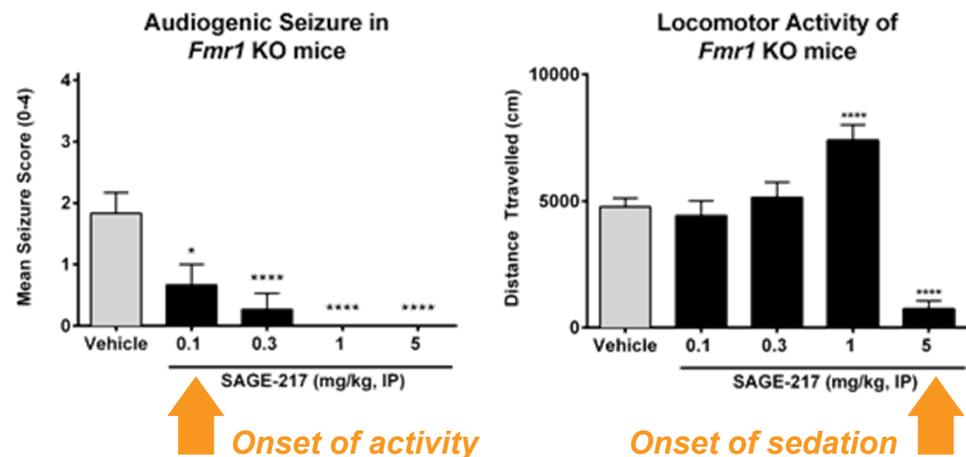


## SAGE-217 Preclinical Data

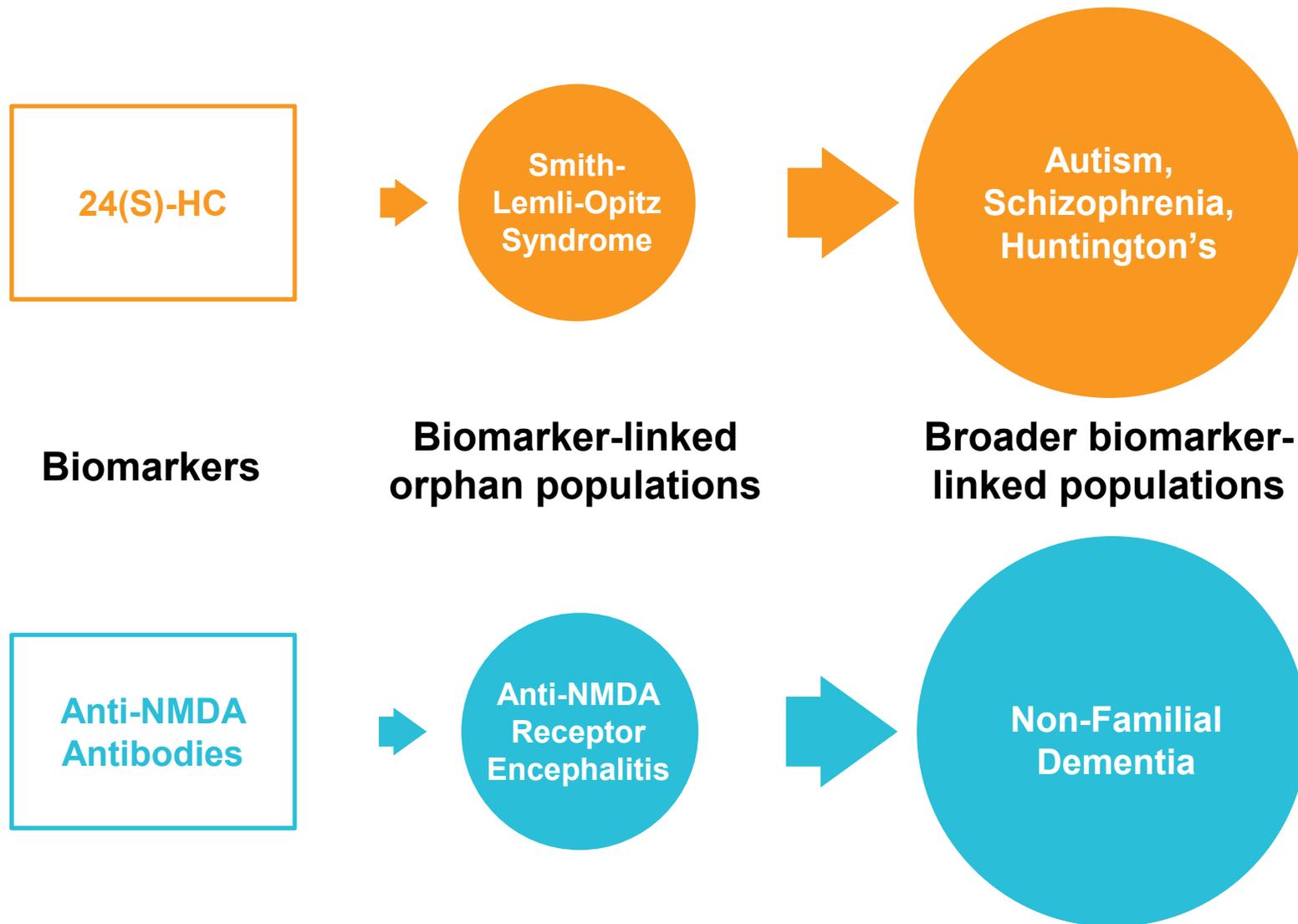
- Robust and dose-related anti-seizure activity in broad variety of acute seizure and chronic epilepsy models
- Showed wide therapeutic index to sedation
- Acute suppression of status epilepticus induced by nerve gas with a related GABA<sub>A</sub> PAM tool compound

## Activity in Genetic Seizure Model

### Model of audiogenic seizures in *Fmr1* knockout mice



# Potential to Explore Biomarker-Defined Disorders of NMDA Hypofunction through Initial Orphan Populations



# Strong Financial Position to Advance Programs

## Q4 2015 Financial Results (as of 12/31/2015)

			FY '15	FY '14
Cash and Cash Equivalents <sup>1</sup>			\$186.8M	\$127.8M
	Q4 '15	Q4 '14	FY '15	FY '14
Research & Development	\$20.4M	\$8.9M	\$69.4M	\$24.1M
General & Administrative	\$8.2M	\$3.4M	\$25.3M	\$9.7M
Net Loss	\$28.6M	\$12.4M	\$94.5M	\$36.1M

<sup>1</sup>Does not include approximately \$140.4M in net proceeds from January 2016 follow-on offering

### Guidance:

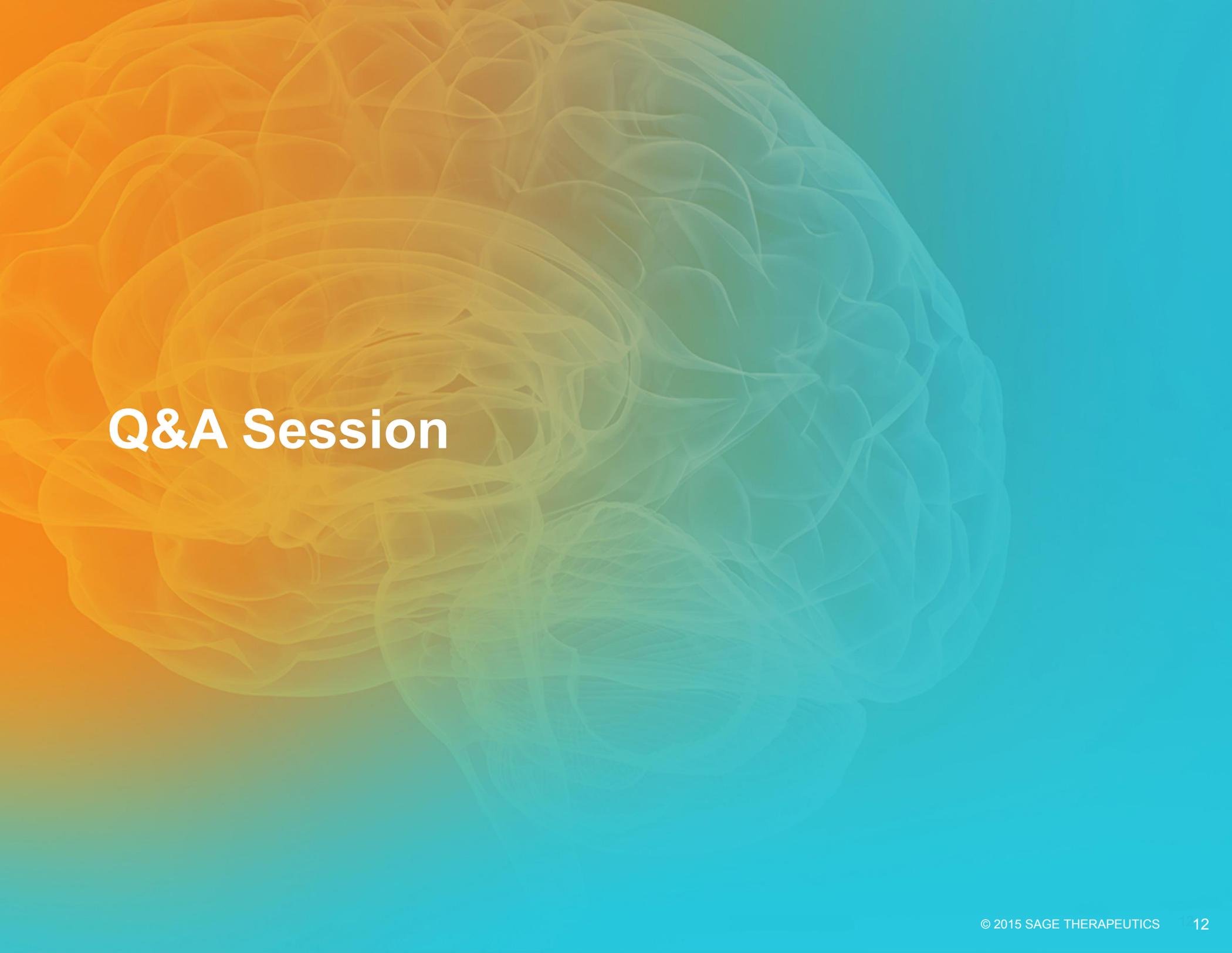
- Based on current operating plan, expect existing cash balance will be sufficient to fund current operations into beginning of 2018

# 2016 Expected Milestones

Program	1H 2016	2H 2016
SAGE-547	Severe postpartum depression <ul style="list-style-type: none"> <li>Phase 2 top-line results</li> </ul>	SRSE <ul style="list-style-type: none"> <li>Phase 3 top-line results</li> <li>NDA planning and pre-commercial activities</li> </ul>
SAGE-217	<b>Phase 1 clinical program results</b>	Essential tremor <ul style="list-style-type: none"> <li>Phase 2 initiation</li> </ul> Orphan epilepsies <ul style="list-style-type: none"> <li>Phase 2 initiation</li> </ul>
SAGE-689		<b>Phase 1 clinical program initiation</b>

# Upcoming Events and Conferences

- **Epilepsy Foundation Pipeline Conference**
  - February 26, 2016
  - San Francisco, CA
- **Cowen and Company 36th Annual Health Care Conference**
  - March 7, 2016, 4:00 PM ET
  - Boston, MA
- **American Chemical Society (ACS) National Meeting & Exposition**
  - March 13 – March 17, 2016
  - San Diego, CA
- **American Academy of Neurology (AAN) 68th Annual Meeting**
  - April 15 – April 21, 2016
  - Vancouver, Canada



# Q&A Session