

DEVELOPING NOVEL THERAPIES
TARGETING NEUROLOGICAL DISEASES



CHANGING CURRENT
TREATMENT PARADIGMS

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Company Highlights

- **Building a Novel, Neurologically-Focused Powerhouse**
- **Risk-Mitigated, Clinical-Stage Assets with Pathway to Approvals in 2017**
- **Significant Market Opportunities Across CNS**
- **Upside Potential from Novel Small Molecule-based Immunoncology Platform**
- **Strong Intellectual Property Position**
- **Leadership Team has Industry-Leading Expertise in Glutamate Research, Backed by Significant Large Cap Pharma-Based Drug Development Expertise**

Accomplished Leadership Team

**Leadership team with over 600 peer-reviewed publications,
50+ collective years of pharmaceutical development / commercialization expertise & leadership**

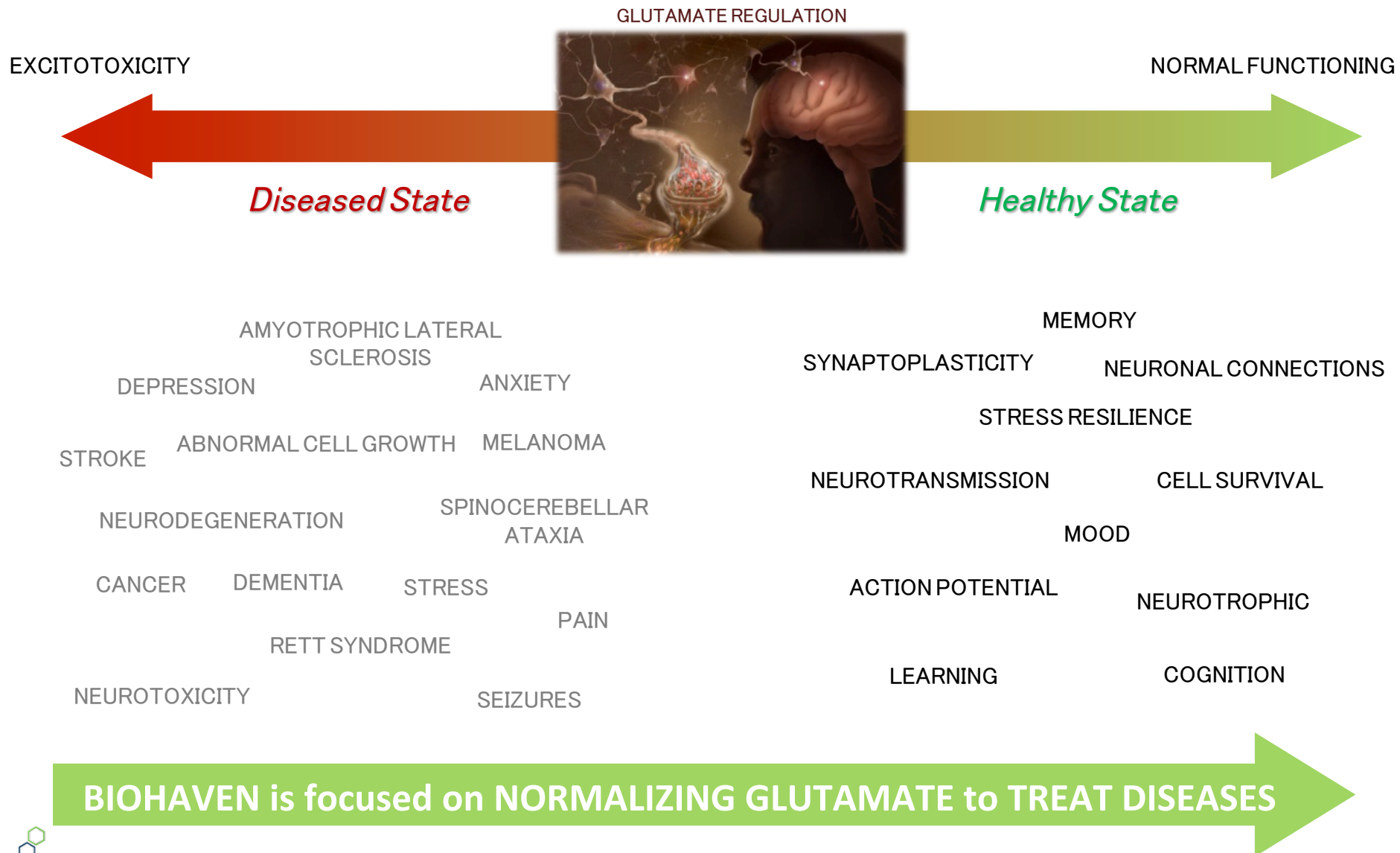
Management Team	Prior Professional Roles			Drug Development / Commercialization Experience			
Vlad Coric, M.D.* <i>Chief Executive Officer</i>	 Bristol-Myers Squibb	 Yale University School of Medicine		 ABILIFY (aripiprazole)	 OPDIVO (nivolumab)	 YERVOY (ipilimumab)	 Daklinza (daklatasvir)
Rob Berman, M.D. <i>Chief Medical Officer</i>	 Bristol-Myers Squibb	 Pfizer	 Yale University School of Medicine	 ABILIFY (aripiprazole)			
John Tilton <i>Chief Commercial Officer</i>	 ALEXION	 Pfizer	 Agouron Pharmaceuticals, Inc.	 SOLIRIS (eculizumab)	 Kanuma (sebelipase alfa)	 Strensiq (asfotase alfa) for injection	 VIRACEPT (nelfinavir mesylate)

Management History of Research and Development Innovation

Led DEVELOPMENT of ZOLOFT (Doogan et al 1994)	ANTI-DEPRESSANT EFFECTS of KETAMINE (Berman et al 2000)	ANTI-ANXIETY properties of the glutamate transport modulator RILUZOLE (Coric et al 2003)	FIRST ANTI-PSYCHOTIC APPROVED in Treatment-Resistant Depression (Berman et al 2007)	FIRST CLINICAL TRIAL of CHECKPOINT INHIBITORS, Opdivo and Yervoy, in GLIOBLASTOMA (Coric, 2013)	FIRST CLINICAL TRIAL in PRODROMAL ALZHEIMER'S DISEASE (Coric et al 2015)
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Board of Directors	Experience / Affiliations	Scientific Advisory Board	Experience / Affiliations
Declan Doogan, M.D. <i>Chairman and Director</i>	 Pfizer  spinifex  PORTAGE PHARMACEUTICALS LTD.  AMARIN SOSEI	John Krystal, M.D. <i>Head of SAB</i>	 Yale  NIH National Institute on Alcohol Abuse and Alcoholism  National Center for PTSD  Yale University
John W. Childs <i>Director</i>	 J.W. Childs ASSOCIATES  THL PARTNERS  Prudential	Maurizio Fava, M.D. <i>SAB Member</i>	 MGH 1811 MASSACHUSETTS GENERAL HOSPITAL  HARVARD MEDICAL SCHOOL
Greg Bailey <i>Director</i>	 MEDIVATION	Gerard Sanacora, M.D. <i>SAB Member</i>	 Yale  Yale University  Stony Brook University
Kam Shah <i>Director</i>	 EY  pwc	Tom Laughren, M.D. <i>SAB Member</i>	 FDA Center for Drug Evaluation and Research  BROWN

The Role of Glutamate – Present in 90% of all brain synapses



Applicable Target Markets

Glutamate Dysfunction Spans Multiple Diseases

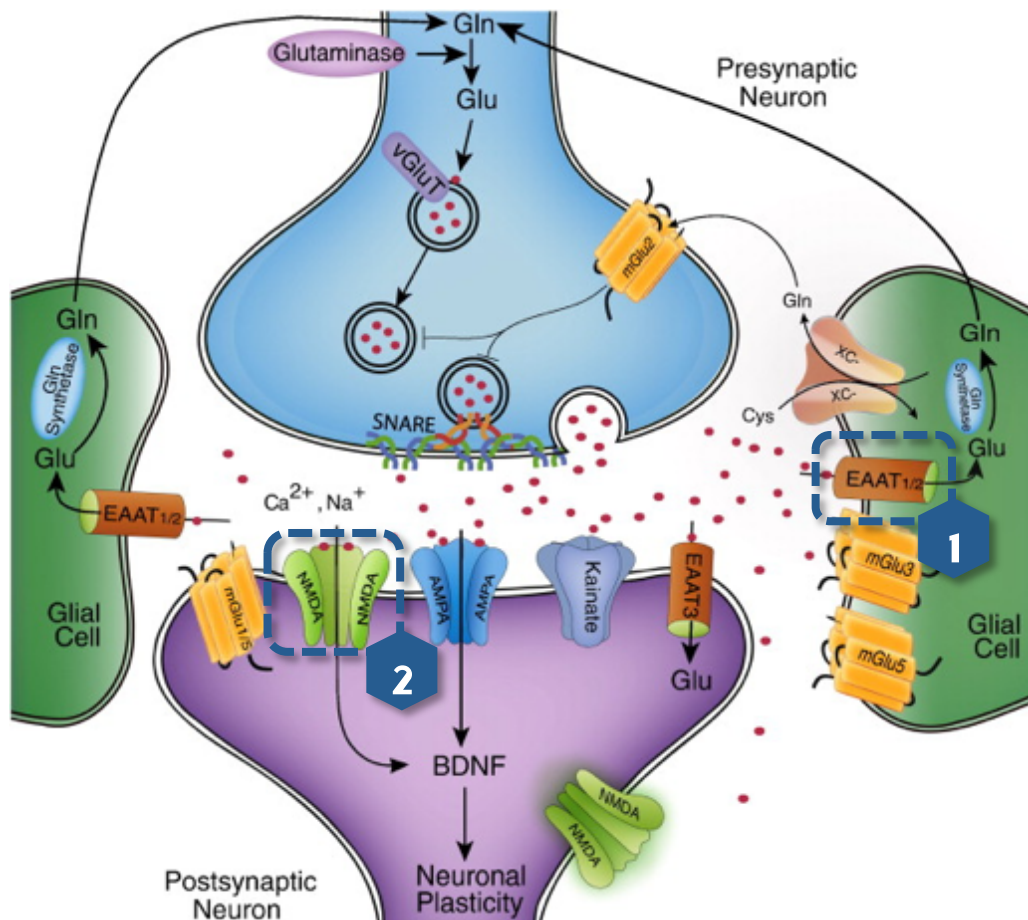
Multi-Billion Dollar Target Market Opportunities



Disease State	U.S. Prevalence	Market Size
Neurodegenerative Disorders	50 M	\$20 B+
Cancer	14 M	\$80 B+
Depression	18 M	\$14 B+
Anxiety Disorders	40 M	\$10 B+

Source: American Cancer Society (<http://www.cancer.org>), National Institute of Health (<http://www.ninds.nih.gov>), National Institute of Mental Health (<http://www.nimh.nih.gov>)

Glutamate Mechanisms of Action in CNS



1

Glutamate transporter modulation

BHV-0223

BHV-4157

2

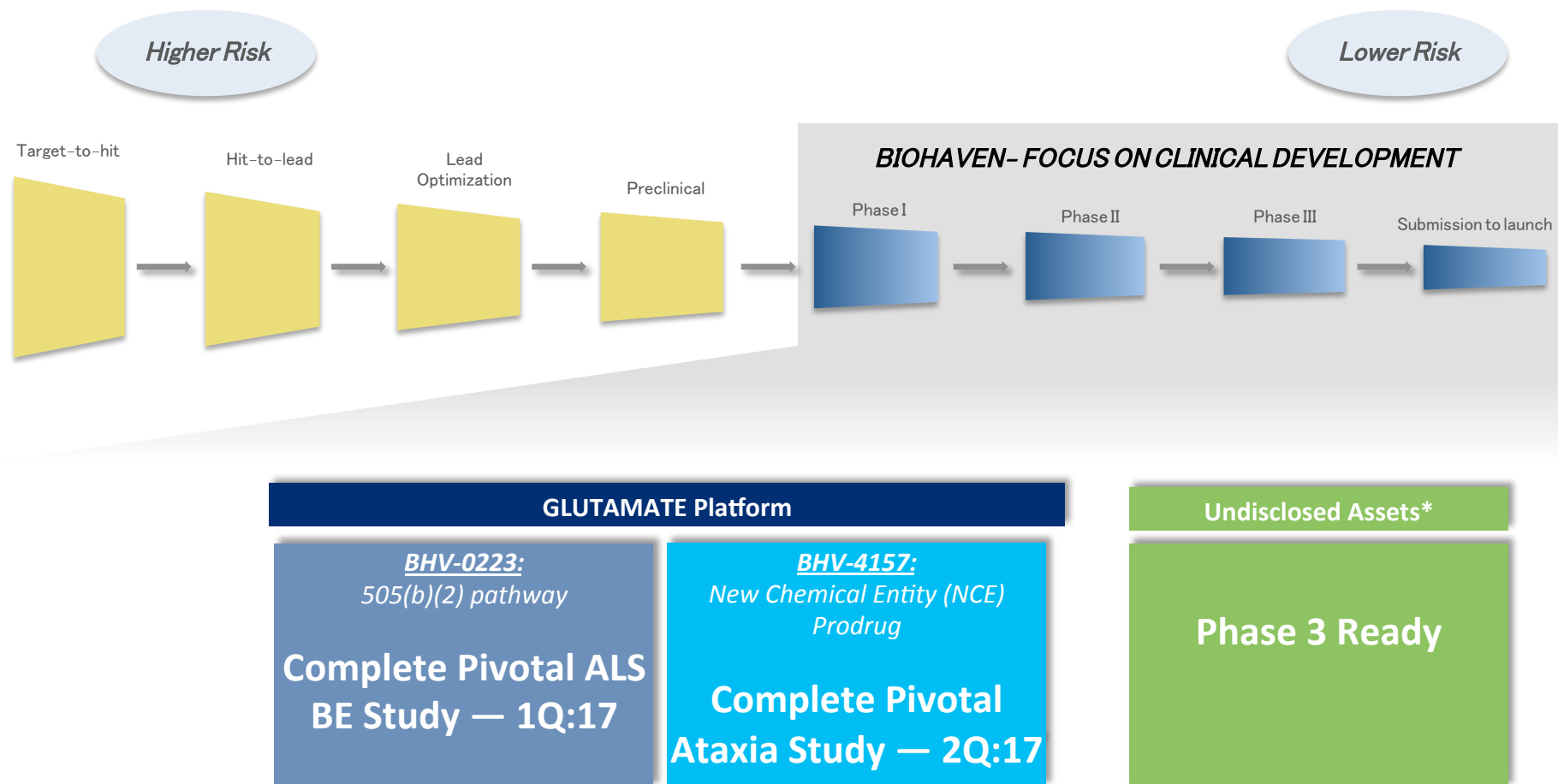
Glutamate NMDA receptor antagonism

Source: Niciu et al., 2012

Proof of concept established in ALS, ataxia, depression and anxiety

Biohaven Neurologic Clinical Portfolio

Focus on *Risk-Mitigated, Clinical-Stage Assets*



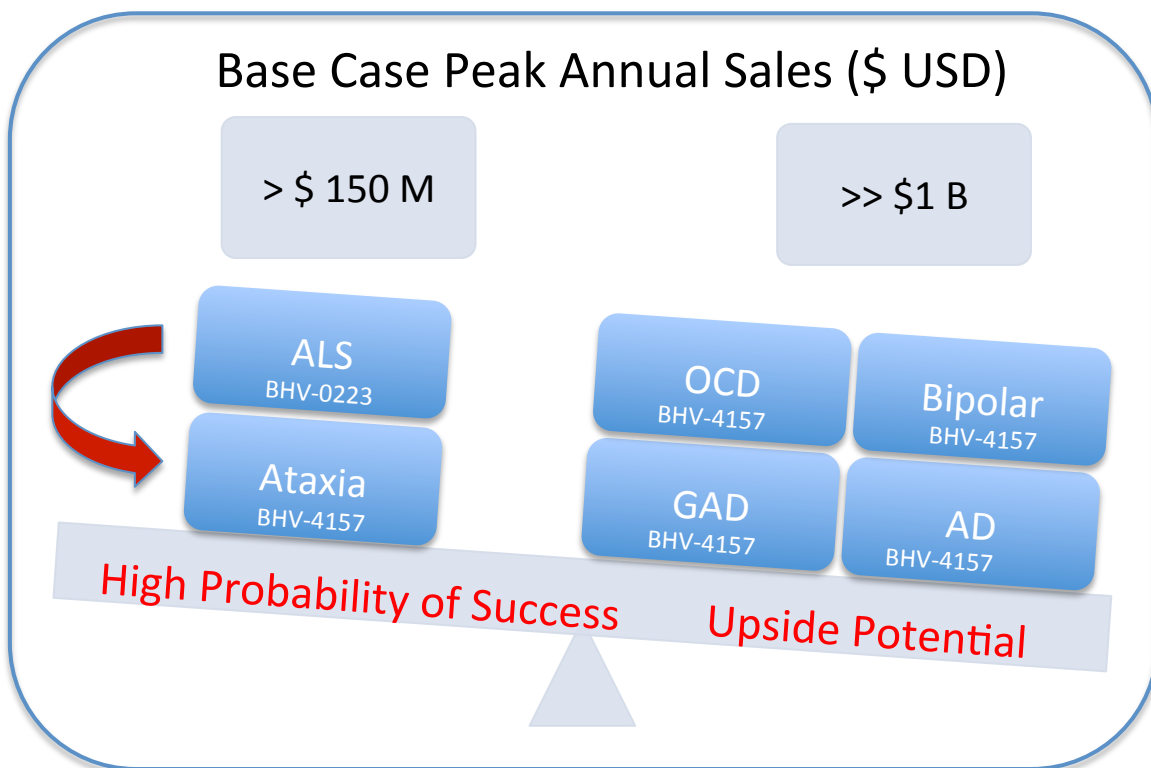
**Biohaven is poised for commercialization as early as 2017
with deep supporting late stage pipeline**

BHV-0223 and BHV-4157 Overview:

Near Term Revenue Opportunities with Tremendous Upside in Many Indications

BHV-0223 and BHV-4157 address limitations of widely used riluzole in treatment of CNS disorders

- **BHV-0223** is a low risk, high probability-of-success **novel sublingual ZYDIS® formulation of riluzole**
 - **505(b)(2) pathway** drives low development risk pathway to kick-start platform
- **BHV-4157 is a New Chemical Entity** prodrug that opens the door to **large market opportunities**
 - 1x daily dosing, optimized bioavailability and PK



Early orphan indications provide lower risk, higher visibility into commercial thesis for our glutamate transporter modulation platform; validates BHV-4157 potential in large CNS Markets markets

BHV-4157 Initial Target Indications

Spinocerebellar Ataxia

Symptoms and Key Considerations:

- Movement disorder, poor coordination, swallowing difficulties, neuropathy, atrophy, etc.
- High unmet need — patients already contacting Biohaven regarding clinical trial
- Potential to be first approved treatment

Addressable Market:

- Orphan disease — Approximately 25,000 patients with Hereditary Spinocerebellar ataxias in US

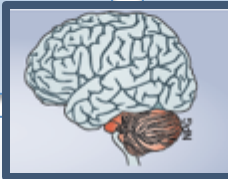
Obsessive Compulsive Disorder

Symptoms and Key Considerations:

- Intrusive thoughts that produce anxiety (obsessions), repetitive behaviors (compulsions)
- OCD has a 12-month prevalence in the United States of 1%, and many have severe symptoms

Addressable Market:

- In Treatment-Resistant OCD, over 300,000 Americans could benefit from treatment with Riluzole ZYDIS



Bipolar Depression

Symptoms and Key Considerations:

- Periods of depressive or manic mood
- 12th leading cause of disability (WHO, 2008)
- Limited treatment options include conventional antidepressants associated with increased cyclicity
- Approved agents (atypical antipsychotics) are associated with wt. gain, sedation and safety issues

Addressable Market:

- ~4.5M Americans (Bipolar Type 1 and 2)— of whom, 10% are clinically depressed

Generalized Anxiety Disorder

Symptoms and Key Considerations:

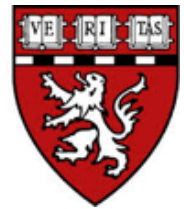
- Chronic or excessive worry, restlessness, fatigue, difficulty concentrating, insomnia
- Impairs ability to function socially or at work
- Irritable bowel-like gastrointestinal issues
- GAD has a 12-month prevalence in the United States of 3%

Addressable Market:

- In Treatment Resistant GAD, 1.5% (> 3.5M) Americans could benefit from Riluzole ZYDIS

Integrated IP Strategy

- **Composition of Matter IP**
 - BHV-4157: NCE with expected 20 years patent protection
 - Undisclosed Asset: NCE with expected 20 years patent protection
- **Exclusive Zydis license from Catalent includes:**
 - World-wide license and new formulation IP for BHV-0223
 - Catalent manufacturing trade secrets
- **Issued Methods of Use Patent from Yale**
- **Divisional Patents and Pending Applications for additional claims:**
 - Dose range
 - Indications (e.g., Post-Traumatic Stress Disorder, Schizophrenia, Bipolar Disorder, Dementia)
 - Sublingual formulation – route of administration and unique PK profile
 - NMDA antagonist and Scopolamine (Harvard IP)
 - Riluzole and Ketamine Prodrugs



Achievements to Date in Neurological Portfolio

REGULATORY

- IND filed and approved for BHV-0223
- Orphan Drug Designation granted in Spinocerebellar Ataxia
- BHV-4157 IND on track for April/May 2016 filing



CLINICAL

- Sublingual BHV-0223 PK study complete demonstrating sublingual absorption (mitigates risk for 4157)
- Lead prodrug (BHV-4157) advanced, representing 6 years of chemistry work
- BHV-0223 and BHV-4157 feasibility studies for Zydis commercial formulation
- BHV-4157 exploratory development work in oncology underway
- Two pending large pharma in-licensing deals underway
- Joint-venture with small molecule immuno-oncology platform






FINANCIAL

- > \$12 M invested to date

Effective and Disciplined Drug Development

Competitive Landscape

Few glutamate agents targeting similar indications

	Route	Company	Clinical Development	Estimated Filing
Rapastinel (GLYX-13) / NRX-1074 <i>tetrapeptide glycine-site partial agonist</i>	IV / oral	 Allergan	<ul style="list-style-type: none"> Naurex in August 2015 for \$560M upfront (plus milestones) Positive results in single dose studies for TRD for rapastinel and NRX-1074 However, failed Phase 2 study for rapastinel. Undisclosed development plan. 	2020
CERC-301 <i>selective NMDA antagonist</i>	oral	 cerecor	<ul style="list-style-type: none"> Commenced TRD trial September 2015 to evaluate higher doses than in the previous failed trial Higher doses associated with hypertension. 	2020
AZD8108 <i>NMDA antagonist</i>	oral	 AstraZeneca	<ul style="list-style-type: none"> AZD8108 is prodrug of AZD6764 Associated with increased BP/dizziness 	2020
S-Ketamine <i>enantiomer of ketamine</i>	Intra-nasal	 Johnson & Johnson	<ul style="list-style-type: none"> Recent data showing beneficial effects of intranasal ketamine on suicidality Full Phase 3 program ongoing in TRD and Suicidality 	2017
Ketamine <i>NMDA antagonist</i>	IV	<i>Academic use</i>	<ul style="list-style-type: none"> Extensively reported efficacy in TRD. 	NA
AV-101 <i>NMDA glycine-site antagonist</i>	oral	 VistaGen [®] Therapeutics	<ul style="list-style-type: none"> Lead compound is prodrug of 7-Cl-KYNA Entering NIH-run Phase 2 study starting ~OCT-2015 (n=25, TRD, dosing x 14 days) 	2020