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 Immuno-oncology 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**

<table>
<thead>
<tr>
<th>Management Team</th>
<th>Prior Professional Roles</th>
<th>Drug Development / Commercialization Experience</th>
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<tbody>
<tr>
<td>Vlad Coric, M.D.*</td>
<td>Bristol-Myers Squibb, Yale University School of Medicine</td>
<td>AKHLYF, OPDIVO, YERVOY (pembrolizumab), Daklinza (daklatasvir)</td>
</tr>
<tr>
<td>Chief Executive Officer</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Rob Berman, M.D.</td>
<td>Bristol-Myers Squibb, Pfizer, Yale University School of Medicine</td>
<td>AKHLYF, OPDIVO, YERVOY (pembrolizumab), Daklinza (daklatasvir)</td>
</tr>
<tr>
<td>Chief Medical Officer</td>
<td></td>
<td></td>
</tr>
<tr>
<td>John Tilton</td>
<td>Alexion, Pfizer, Agouron Pharmaceuticals, Inc.</td>
<td>SOLIRIS, Kanuma, Strensis (ensilutem mesilate), VIRACEREP (enilutem mesilate)</td>
</tr>
<tr>
<td>Chief Commercial Officer</td>
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**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 APROVED in Treatment-Resistant Depression** (Berman et al 2007)
- **FIRST CLINICAL TRIAL of CHECKPOINT INHIBITORS, Opdivo and Yervoy, in GIOBLASTOMA** (Coric, 2013)
- **FIRST CLINICAL TRIAL in PRODROMAL ALZHEIMER'S DISEASE** (Coric et al 2015)

<table>
<thead>
<tr>
<th>Board of Directors</th>
<th>Experience / Affiliations</th>
<th>Scientific Advisory Board</th>
<th>Experience / Affiliations</th>
</tr>
</thead>
<tbody>
<tr>
<td>Declan Doogan, M.D.</td>
<td>Pfizer, MEDIVATION</td>
<td>John Krystal, M.D.</td>
<td>Yale, NIH</td>
</tr>
<tr>
<td>Chairman and Director</td>
<td></td>
<td>Head of SAB</td>
<td>National Institute on Alcohol Abuse and Alcoholism</td>
</tr>
<tr>
<td>John W. Childs</td>
<td>J.W. CHILDS ASSOCIATES</td>
<td>Maurizio Fava, M.D.</td>
<td>Massachusetts General Hospital</td>
</tr>
<tr>
<td>Director</td>
<td></td>
<td>SAB Member</td>
<td>Harvard Medical School</td>
</tr>
<tr>
<td>Greg Bailey</td>
<td>Prudential</td>
<td>Gerard Sanacora, M.D.</td>
<td>Yale</td>
</tr>
<tr>
<td>Director</td>
<td></td>
<td>SAB Member</td>
<td>Stony Brook University</td>
</tr>
<tr>
<td>Kam Shah</td>
<td>EY, PWC</td>
<td>Tom Laughren, M.D.</td>
<td>FDA, BROWN</td>
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<td>Director</td>
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*Company executive also serves on Board of Directors.*
The Role of Glutamate – Present in 90% of all brain synapses

GLUTAMATE REGULATION

EXCITOTOXICITY

Diseased State

Healthy State

NORMAL FUNCTIONING

AMOTROPHIC LATERAL SCLEROSIS
DEPRESSION
STROKE
NEURODEGENERATION
CANCER
NEUROTOXICITY

SYNAPTOPLASTICITY
MEMORY
NEURONAL CONNECTIONS
STRESS RESILIENCE
NEUROTRANSMISSION
CELL SURVIVAL
MOOD
ACTION POTENTIAL
LEARNING
COGNITION

BIOHAVEN is focused on NORMALIZING GLUTAMATE to TREAT DISEASES
### Multi-Billion Dollar Target Market Opportunities

<table>
<thead>
<tr>
<th>Disease State</th>
<th>U.S. Prevalence</th>
<th>Market Size</th>
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<tbody>
<tr>
<td>Neurodegenerative Disorders</td>
<td>50 M</td>
<td>$20 B+</td>
</tr>
<tr>
<td>Cancer</td>
<td>14 M</td>
<td>$80 B+</td>
</tr>
<tr>
<td>Depression</td>
<td>18 M</td>
<td>$14 B+</td>
</tr>
<tr>
<td>Anxiety Disorders</td>
<td>40 M</td>
<td>$10 B+</td>
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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

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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

GLUTAMATE Platform

**BHV-0223:** 505(b)(2) pathway

Complete Pivotal ALS BE Study — 1Q:17

**BHV-4157:** New Chemical Entity (NCE) Prodrug

Complete Pivotal Ataxia Study — 2Q:17

Undisclosed Assets*

Phase 3 Ready

(*in-licensing in process)
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

Base Case Peak Annual Sales ($ USD)

- > $150 M
- >> $1 B

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
### 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

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### 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

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### 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

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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

<table>
<thead>
<tr>
<th>Route</th>
<th>Company</th>
<th>Clinical Development</th>
<th>Estimated Filing</th>
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</table>
| IV / oral        | **Rapastinel (GLYX-13) / NRX-1074 tetrapeptide glycine-site partial agonist** | - 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             |
| Oral             | **CERC-301 selective NMDA antagonist** | - Commenced TRD trial September 2015 to evaluate higher doses than in the previous failed trial  
- Higher doses associated with hypertension. | 2020             |
| Oral             | **AZD8108 NMDA antagonist** | - AZD8108 is prodrug of AZD6764  
- Associated with increased BP/dizziness | 2020             |
| Intra-nasal      | **S-Ketamine enantiomer of ketamine** | - Recent data showing beneficial effects of intranasal ketamine on suicidality  
- **Full Phase 3 program ongoing in TRD and Suicidality** | 2017             |
| IV               | **Ketamine NMDA antagonist** | - Extensively reported efficacy in TRD. | NA               |
| Oral             | **AV-101 NMDA glycine-site antagonist** | - Lead compound is prodrug of 7-CI-KYNA  
- Entering NIH-run Phase 2 study starting ~OCT-2015 (n=25, TRD, dosing x 14 days) | 2020             |