



HIGHLAND  
THERAPEUTICS

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For Immediate Release:

## **HIGHLAND THERAPEUTICS ANNOUNCES POSITIVE PIVOTAL ADHD TRIAL RESULTS FOR ITS INVESTIGATIONAL DRUG CANDIDATE BENJORNA™**

- Benjorna™ met the primary endpoint – composite score from 8am to 8pm based on SKAMP
- Benjorna™ met the key secondary endpoint – improved functioning during the morning based on PREMB-R AM subscale
- Benjorna™ also achieved statistically significant improvement in functioning during the evening, based on PREMB-R PM subscale
- Benjorna™ was well tolerated, with majority of side effects generally mild and resolved during the course of treatment
- NDA submission expected in mid-2016
- Two new drug candidates to be advanced into pivotal trials in 2H-2016

TORONTO, Canada, April 5, 2016—Highland Therapeutics Inc.'s wholly owned subsidiary, Ironshore Pharmaceuticals & Development, Inc. ("Ironshore"), today announced positive clinical data from the first of two Phase III pivotal trials of its investigational drug product, Benjorna™ (delayed-release and extended-release methylphenidate capsules). Benjorna™, formerly referred to as HLD-200, is under development as a potential new option for physicians treating patients with Attention Deficit Hyperactivity Disorder (ADHD). In the clinical study (HLD200-107), which included 153 pediatric patients, those who were randomized to receive Benjorna™ demonstrated a statistically significant improvement compared to those patients who received placebo ( $p=0.01$ ), based on a composite measure from 8am through to 8pm on the Swanson, Kotkin, Agler, M-Flynn and Pelham (SKAMP) Rating Scale, the study's primary endpoint.

Patients randomized to the Benjorna™ treatment arm also demonstrated improved functioning during the morning routine as measured by the PREMB-R (Parent Rating of Evening and Morning Behavior-Revised) morning (AM) subscale, which was the study's key secondary endpoint ( $p<0.001$ ). The potential for Benjorna™ to improve functioning during the morning

routine will be evaluated further in a second pivotal study (HLD200-108), which is currently ongoing with results expected in the second quarter of 2016.

In the HLD200-107 study, the Benjorna™ treatment group also achieved statistically significant improvement, compared with placebo, in functioning in the evening as measured by the PREMB-R evening (PM) subscale ( $p < 0.001$ ). The extended duration of clinical effect was previously observed in a prior Phase III study, conducted in 2014, in which patients receiving evening-dosed Benjorna™ showed improved functioning through to 8pm the following day, the last time point measured.

Commenting on the HLD200-107 results, Dr. Bev Incedon, Ironshore's Executive Vice President of Research and Development said, "The positive results from this pivotal trial are just the latest evidence that the Company has taken another step towards its stated goal of bringing to patients and physicians a new medicine that, if approved by the FDA, could become the standard of care for the treatment of ADHD. We currently believe that there is something unique happening at the dopamine neurotransmission level that may be attributed to a unique absorption profile, which will be the subject of an upcoming study led by a team of thought leaders. This work will be important for the medical community to consider and will surely deepen our appreciation of the potentially critical role that pharmacokinetics have on the effectiveness of stimulant medications."

Dr. Randy Sallee, Chief Medical Officer stated, "As a practicing child and adolescent psychiatrist for more than 35 years, I believe the most impressive result from the clinical trial is the robust and consistent effect reported across the entire day; from waking, through the academic day and into the evening period. Importantly, in the HLD200-107 study, there was no difference in the number of sleep-related side effects between the Benjorna™ and placebo groups."

David Lickrish, President and Chief Executive Officer stated, "The last 12 months have been transformational for Highland, which is emerging as a fully integrated pharmaceutical company and a future leader in neuroscience, including ADHD. With these positive results in hand, we will not only continue to invest in our business as we become more vertically integrated, but we will accelerate that pace of activity so that the requisite teams are in place to support the launch of this product upon FDA approval. In addition to these milestones, we expect to make several other near-term announcements regarding our rapidly expanding pipeline."

## **Pipeline Developments**

Ironshore intends to advance two new drug candidates into pivotal trials in the second half of 2016: HLD-900, an amphetamine-based product for the treatment of Binge Eating Disorder (BED) which was the subject of an End of Phase 2 (EOP2) meeting held with the U.S. Food and Drug Administration in February 2016, and HLD-100, which is under development as an amphetamine-based treatment for patients with ADHD.

### **About the HLD200-107 Study**

Initiated in July 2015, the HLD200-107 study was a Phase III, multicenter, open-label, treatment-optimized, double-blind, randomized, placebo-controlled, forced-withdrawal, parallel group study to evaluate the safety and efficacy of evening-dosed Benjorna™ in children aged 6-12 with ADHD in a laboratory classroom setting. A total of 153 patients were randomized across seven sites in the US.

### **About the HLD200-108 Study**

Initiated in August 2015, trial HLD200-108 is a Phase III, multicenter, double-blind, randomized, placebo-controlled, parallel group study to evaluate the safety and efficacy of evening-dosed Benjorna™ on post-waking, early morning function in children aged 6-12 with ADHD. The study will randomize approximately 150 patients at 22 centers across the US.

### **About Highland Therapeutics Inc.**

Highland Therapeutics Inc. is a specialty pharmaceutical company that, through its wholly owned subsidiary Ironshore Pharmaceuticals & Development, Inc., is leveraging its proprietary technology, DELEXIS®, to optimize the delivery of previously approved drug products. The Company's lead product candidates, HLD-200 and HLD-100, are novel formulations of the psychostimulants (methylphenidate and amphetamine, respectively) used to treat ADHD and are being developed to address a prevalent unmet medical need in the treatment of the disease – inadequate symptom control during the morning routine. Intended for nighttime dosing, DELEXIS® is designed to provide a consistent delay in the initial release of the active drug, followed by a period of extended release; with the objective of providing control of ADHD symptoms immediately upon waking and throughout the day.

Highland Therapeutics Inc. is a client of MaRS Discovery District's Health Venture Services group, which provides advisory services, connections to talent, customer & capital networks, and market intelligence to high-impact, Ontario-based life sciences ventures, helping them commercialize their ideas and build globally competitive companies.

For further information, please visit the Company's website at [www.highlandtherapeutics.com](http://www.highlandtherapeutics.com), or contact:

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### **Forward-Looking Statements**

This press release contains forward-looking information, which reflects Highland's current expectations regarding future events. Forward-looking information is based on a number of assumptions and is subject to a number of risks and uncertainties, many of which are beyond Highland's control that could cause actual results and events to differ materially from those that are disclosed in or implied by such forward-looking information. These forward-looking statements are made as of the date of this press release and, except as expressly required by applicable law, Highland assumes no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise.