



**IRONSHORE**

PHARMACEUTICALS & DEVELOPMENT, INC.

[www.ironshorepharma.com](http://www.ironshorepharma.com)

For Immediate Release:

## **IRONSHORE PHARMACEUTICALS ANNOUNCES POSITIVE HLD100 RESULTS FROM OPEN-LABEL TOLERABILITY STUDY IN PEDIATRIC ADHD PATIENTS**

- Results support HLD100's potential to control symptoms and improve functioning during both the early morning routine and bedtime routine
- SDSC scores suggest no adverse impact on sleep with HLD100
- Ironshore intends to initiate two pivotal studies in 2017 in support of a New Drug Application with a differentiated label

George Town, Grand Cayman, April 12, 2017 – Ironshore Pharmaceuticals & Development, Inc. (“Ironshore”), a wholly owned subsidiary of Highland Therapeutics Inc., today announced that it has presented to the U.S. Food and Drug Administration (“FDA”) the results from an open-label clinical trial that was conducted to investigate the tolerability and efficacy of HLD100 – a novel delayed-release, extended-release (“DR/ER”) formulation of amphetamine currently under development as a potential new treatment option for patients with Attention-Deficit/Hyperactivity Disorder (“ADHD”).

HLD100 was well tolerated with no serious adverse events noted during the study. The majority of side effects were mild or moderate in severity and resolved during the course of the treatment. Given the novelty of HLD100's evening administration, and to probe for any potential impact on sleep, the Sleep Disturbance Scale for Children (“SDSC”) was used. Importantly, the results showed median values for the SDSC total score and all subscale scores either the same or lower (improved) than at baseline.

“The dedicated employees at Ironshore have worked tirelessly over the past several years to develop a novel medication that may provide improved clinical outcomes for ADHD patients and their caregivers,” said David Lickrish, Ironshore's Chief Executive Officer. “With the FDA's PDUFA Action Date for our other investigational ADHD drug, HLD200 (methylphenidate DR/ER), coming later this year, Ironshore is reaffirming its commitment to families and developing an additional treatment option. Similar to HLD200, which was evaluated in 10 clinical

studies, including two pivotal trials, our amphetamine program is equally ambitious and will evaluate HLD100 in two large pivotal trials, which may help to inform the public regarding its unique value proposition and its potential to become a first-line treatment option in ADHD, if approved.”

The HLD100-103 clinical trial enrolled 22 pediatric patients (ages 6 to 12) in a single center in the US. Patients were titrated over a five-week period to evaluate (i) optimal dosage strength and (ii) optimal clinical effects. Similar to the HLD200 (methylphenidate DR/ER) pivotal studies, Ironshore used the Before School Functioning Questionnaire (“BSFQ”) and the Parent Rating of Evening and Morning Behavior – Revised (“PREMB-R”) morning (“AM”) and evening (“PM”) subscales, as well as the ADHD-RS-IV scale as assessment tools. The Weiss Functional Impairment Rating Scale (“WFIRS”) was also used.

The average dosage strength in the study was 25.9 mg, which is below the recommended maximum dose for other amphetamine formulations. Importantly, a clinical effect was observed upon awakening that lasted through to the evening bedtime routine period. Our pivotal studies will further evaluate the observed preliminary signals that HLD100 may have a long duration of action without any discernible rebound effects that are sometimes reported with stimulant medications<sup>1</sup>.

At baseline, ADHD-RS-IV, BSFQ, PREMB-R AM and PREMB-R PM scores were 36.1, 28.6, 5.8 and 14.1, respectively. Following five weeks of treatment, these scores improved to 13.3, 8.8, 1.5 and 5.7, respectively; representing an improvement of 63%, 68%, 74% and 60%, respectively. On the WFIRS scale, scores improved from 48.5 at baseline to 27.7 at the end of the study, representing a 43% improvement in functioning.

The results of the HLD100-103 study were reviewed at an End of Phase 2 meeting with the FDA. Based on feedback we received at the meeting with FDA, Ironshore is pursuing a clinical program for HLD100 that will include two pivotal Phase 3 studies. Results from these studies, if successful, may demonstrate replication of effect during specific time periods, which could result in a differentiated drug label. Ironshore intends to initiate the pivotal trials in the third quarter of 2017, with a New Drug Application expected in 2018.

Dr. Randy Sallee, Ironshore’s Chief Medical Officer and author of more than 100 journal articles on ADHD said, “The results from the HLD100-103 study are better than anticipated and further validate Ironshore’s investment in pursuing a new approach to the treatment of ADHD. As a science-driven organization, we are pleased to continue to build on the development path of HLD200, which I believe is the first product in clinical trials to demonstrate significant

improvements in each of early morning, late afternoon and evening impaired functioning with a single dose of a long-acting stimulant in children with ADHD.”

1. Lopez, Frank A., *et al. ADHD Symptom Rebound and Emotional Lability With Lisdexamfetamine Dimesylate in Children Aged 6 to 12 Years*. Journal of Attention Disorders, 2017 Volume 21.

### **About Ironshore Pharmaceuticals & Development, Inc.**

Ironshore Pharmaceuticals & Development, Inc., a wholly owned subsidiary of Highland Therapeutics Inc., is a pharmaceutical company that is leveraging its proprietary technology, DELEXIS®, to optimize the delivery of previously approved drug products.

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 contact:**

Nelson F. Isabel  
Chief Financial Officer  
(647) 260-7875

### **Forward-Looking Statements**

This press release contains forward-looking information, which reflects Ironshore’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 Ironshore’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, Ironshore assumes no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise.