Ironwood Pharmaceuticals Announces FDA Fast Track Designation for Praliciguat for the Treatment of Heart Failure with Preserved Ejection Fraction (HFpEF)

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– Praliciguat Phase II trial in HFpEF continues to enroll patients with topline data expected in the second half of 2019 –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 13, 2018-- Ironwood Pharmaceuticals, Inc. (NASDAQ: IRWD), a commercial biotech company, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track Designation for praliciguat (IW-1973) for the treatment of patients with heart failure with preserved ejection fraction (HFpEF). Praliciguat is an investigational, orally administered soluble guanylate cyclase (sGC) stimulator currently in Phase II clinical trials.

“An ever-increasing number of people are suffering from HFpEF, a disease characterized by exercise intolerance, frequent hospitalizations, and increased risk of death, yet there are no approved treatment options,” said Christopher Wright, M.D., Ph.D., senior vice president of global development and chief development officer of Ironwood. “We believe praliciguat has the potential to be a new treatment option for these patients and are researching its ability to provide multi-dimensional impact on this disease by increasing tissue blood flow and decreasing cardiac vascular inflammation and fibrosis. The Fast Track Designation underscores the seriousness of this disease and that praliciguat has potential to address unmet needs in HFpEF. We look forward to working closely with the FDA to rapidly progress the development of praliciguat for the treatment of HFpEF.”

The FDA grants Fast Track Designation to facilitate the development and expedite the review of drugs that have the potential to treat serious or life-threatening diseases. A drug granted Fast Track Designation is eligible for several benefits, including more frequent meetings with and communications from the FDA and potentially for Rolling Review of the New Drug Application (NDA) and Priority Review if relevant criteria are met.[1]

Ironwood is currently enrolling patients in a randomized, double-blind, placebo-controlled Phase II trial evaluating praliciguat for the potential treatment of HFpEF. Ironwood expects to enroll approximately 175 patients into the Phase II trial, which is designed to evaluate the safety and efficacy of praliciguat in patients with HFpEF. Topline data from this study are expected in the second half of 2019. Further details about the trial can be found at clinicaltrials.gov using the identifier number NCT03254485.

About Heart Failure with Preserved Ejection Fraction

Heart failure with preserved ejection fraction (HFpEF) is a form of heart failure that occurs when the left ventricle becomes stiff and its ability to relax is reduced, causing the heart to be unable to fill with blood sufficiently. HFpEF affects an increasing number of people in the developed world, likely due to an increase in common risk factors such as old age, hypertension, and obesity.

About Praliciguat

Praliciguat (IW-1973), an investigational, oral, once-daily soluble guanylate cyclase (sGC) stimulator, is being studied in patients with diabetic nephropathy and in patients with heart failure with preserved ejection fraction (HFpEF). Diabetic nephropathy affects an estimated eight million Americans and 20 to 40 percent of all diabetic patients worldwide. It is the leading cause of end-stage renal disease. Currently available products do not treat the underlying pathophysiology of the disease or fully address the needs of this patient population. HFpEF affects an estimated three million Americans and 40 to 70 percent of heart failure patients worldwide. It is a highly symptomatic condition with high rates of morbidity and mortality that can cause insufficient delivery of oxygen to the tissues, fluid in the lungs and edema of the extremities, causing patients to be short of breath and have compromised exercise tolerance. There are no approved therapies to treat HFpEF.

Currently in Phase II development for diabetic nephropathy and for HFpEF, praliciguat has the potential to address the underlying causes of these devastating diseases by improving nitric oxide (NO) signaling, which may improve vascular and metabolic function and decrease the inflammatory and fibrotic consequences associated with these diseases.

About Ironwood's sGC Program

As a pioneering expert in cyclic GMP (cGMP), Ironwood is building on its success with linaclotide, which stimulates guanylate cyclase-C in the intestine, to develop a pipeline of soluble guanylate cyclase (sGC) stimulators. sGC plays an important role in regulating diverse physiological processes; dysregulation of sGC may play a role in multiple serious diseases. Ironwood's sGC stimulators are believed to harness the nitric oxide (NO)/sGC/cGMP pathway by working synergistically with NO to improve blood flow and metabolism and decrease inflammation and fibrosis.

Ironwood is advancing praliciguat (IW-1973) for the potential treatment of diabetic nephropathy and of heart failure with preserved ejection fraction (HFpEF). Olinciguat (IW-1701) is being developed for the potential treatment of achalasia and of sickle cell disease. In addition, Ironwood has a pipeline of other sGC stimulators in pre-clinical development.

About Ironwood Pharmaceuticals

Ironwood Pharmaceuticals (Nasdaq: IRWD) is a commercial biotechnology company focused on creating medicines that make a difference for patients, building value for our fellow shareholders, and empowering our passionate team. We are currently commercializing two innovative primary care products: linaclotide, the U.S. branded prescription market leader for adults with irritable bowel syndrome with constipation (IBS-C) or chronic idiopathic constipation (CIC), and lesinurad, which is approved to be taken with a xanthine oxidase inhibitor (XOI), or as a fixed-dose combination with allopurinol, for the treatment of hyperuricemia associated with gout. We are also advancing a pipeline of innovative product candidates in areas of significant unmet need, including persistent gastroesophageal reflux disease, diabetic nephropathy, heart failure with preserved ejection fraction,

achalasia and sickle cell disease. Ironwood was founded in 1998 and is headquartered in Cambridge, Mass. For more information, please visit www.ironwoodpharma.com or www.twitter.com/ironwoodpharma; information that may be important to investors will be routinely posted in both these locations.

Forward-Looking Statements

This press release contains forward-looking statements. Investors are cautioned not to place undue reliance on these forward-looking statements, including statements about Ironwood's sGC program and the clinical program for praliciguat, including the design, size, and scope of the Phase II clinical trial; the mechanism of action of praliciguat; the size of the potential patient population and treatment options for HFpEF; the data to be generated from the Phase II clinical trial and the timing of such data; the cause of the disease and the symptoms suffered by the potential patient population; and praliciguat as a potential treatment for HFpEF. Each forward-looking statement is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement. Applicable risks and uncertainties include those related to the risk that we are unable to enroll as many patients in the clinical study or complete the Phase II clinical trial on the same timeline as we currently anticipate; the risk that the data from the clinical trial may not be available when we currently anticipate them or do not demonstrate the results we expect, including with respect to efficacy, safety and tolerability; the risk that the Phase II clinical trial needs to be discontinued for any reason, including safety, enrollment, manufacturing or economic reasons; the patient population is not as large as we presently estimate; the effectiveness of development and commercialization efforts; preclinical and clinical development, manufacturing and formulation development; the risk that findings from our completed nonclinical and clinical studies may not be replicated in later studies; decisions by regulatory authorities; the risk that we may never get sufficient patent protection for praliciguat or that we are not able to successfully protect such patents; the outcomes in legal proceedings to protect or enforce the patents relating to praliciguat; developments in the intellectual property landscape; challenges from and rights of competitors or potential competitors; the risk that our planned investments do not have the anticipated effect on our business or the praliciguat program; and those risks listed under the heading "Risk Factors" and elsewhere in Ironwood's Quarterly Report on Form 10-Q for the quarter ended June 30, 2018, and in our subsequent SEC filings. These forward-looking statements (except as otherwise noted) speak only as of the date of this press release, and Ironwood undertakes no obligation to update these forward-looking statements.

[1] "Fast Track." US Food and Drug Administration. https://www.fda.gov/ForPatients/Approvals/Fast/ucm405399.htm. Published January 4, 2018. Accessed September 12, 2018.

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