Innocrin Pharmaceuticals, Inc. Initiates Phase 2 Castration-Resistant Prostate Cancer (CRPC) Study in Men Who Have Failed Enzalutmaide or Abiraterone

- *Innovative Correlative Study to Assess VT-464 Benefit in CRPC Patients Refractory to Abiraterone or Enzalutamide*

RESEARCH TRIANGLE PARK, NC, May 27 2015 – Innocrin Pharmaceuticals, Inc., a clinical-stage pharmaceutical company developing small-molecule CYP17 lyase-selective inhibitors for the treatment of hormonally-dependent breast and prostate cancers resistant to standard hormonal therapy, today announced the initiation of a new Phase 2 study: Once-daily Oral VT-464 in Patients With CRPC Progressing on Enzalutamide or Abiraterone (https://clinicaltrials.gov/ct2/show/NCT02445976). The trial is led by Howard Scher, MD, Chief of Genitourinary Oncology Service at Memorial Sloan Kettering, who is the recipient of a prestigious $1 million Challenge Award from the Prostate Cancer Foundation (PCF). Dr. Scher is a recognized leader of a 'personalized medicine' approach for CRPC, the goal being to deliver the right drug to patients at the right time.

William Moore, PhD, Innocrin Chief Executive Officer stated "This study will help inform why 30 to 40% of men with CRPC do not initially respond to the currently approved drugs, why other men become resistant to them, and which patients will most likely benefit from VT-464. The study will also inform whether the tumors of these patients express reported biomarkers of resistance to abiraterone and enzalutamide, including androgen receptor (AR) mutations and splice variants such as AR-v7, or the glucocorticoid receptor, and whether or not, similar to preclinical models, they will respond to VT-464."

About VT-464

VT-464 is a once-daily oral medication given without prednisone that may represent an improved therapy for men with CRPC. VT-464 selectively inhibits CYP17 lyase, a target of abiraterone, and blocks the AR, similar to enzalutamide. There is a strong and growing body of preclinical and clinical evidence that some abiraterone- or enzalutamide-resistant patients will respond to VT-464 treatment. Resistance to abiraterone, enzalutamide or both represents a major unmet medical need due to the widespread and growing use of these new agents, and their high cross-resistance (i.e., men with CRPC who have been treated with either abiraterone or enzlutamide are unlikely likely to respond to the other).

VT-464 may also have significant commercial potential in the treatment of breast cancer due to its ability to block biosynthesis of both androgen and estrogen through inhibition of CYP17 lyase, in addition to its AR antagonist activity. Most breast cancers that express the estrogen receptor (e.g., are ER+) or are triple-negative (e.g., do not express the estrogen, progesterone, or Her-2 receptors) also express the AR. It is thought that the AR may stimulate tumor growth in patients whose tumors are triple-negative or are ER+ but have become resistant to ER-directed therapies such as aromatase inhibitors or tamoxifen. Recent clinical efficacy was demonstrated with enzalutamide in triple-negative breast cancers validating the role of the AR in tumor progression in these patients. Recent preclinical

study results have confirmed that VT-464 potently blocks the growth of both ER+ and AR+ triple-negative breast cancer cells.

About Prostate Cancer
Prostate cancer is the second most common form of cancer affecting men in the United States: an estimated one in six will be diagnosed with prostate cancer in his lifetime. The American Cancer Society estimates that approximately 240,000 new cases of prostate cancer will be diagnosed and about 30,000 men will die of the disease this year, and that approximately two million men in the U.S. currently count themselves among prostate cancer survivors.

About Breast Cancer
Each year over 230,000 new cases of breast cancer are diagnosed in the United States, with almost 40,000 deaths attributable to the disease. While estrogen deprivation is currently the standard of care for postmenopausal women with hormone receptor-positive breast cancer, the majority of patients eventually develop resistance. An exciting new treatment biomarker may be the AR, which is believed to be more widely expressed in breast cancer (in 75%-85%) than the ER. Though patients with the ER+/AR+ subtype comprise ~75% of all metastatic BC cases, the most significant unmet need lies in the triple-negative subtype, a population that might respond well to anti-androgen therapies as evidenced by recent results from a Phase 2 study using enzalutamide.

About Innocrin Pharmaceuticals, Inc. (www.innocrinpharma.com)
Innocrin discovers and develops novel, best-in-class oral inhibitors of CYP17 lyase, a validated enzyme target for the treatment of castration-resistant prostate cancer (CRPC). VT-464 and structurally-related classes of CYP17 inhibitors are wholly owned by Innocrin. CYP17 lyase inhibitors may also have high commercial potential for the treatment of breast cancer as well as non-oncologic syndromes that are due to hormonal excess including endometriosis, polycystic ovary syndrome and congenital adrenal hyperplasia. Innocrin's investors include Novartis Venture Fund, Eshelman Ventures, Lilly Ventures, Hatteras Venture Partners, Intersouth Partners, Lurie Holdings, and Astellas Venture Management.