Innocrin Pharmaceuticals Presents Data from the Ongoing Phase 2 Trial of Seviteronel in Estrogen Receptor-positive or Triple-negative Breast Cancer (CLARITY-01) at the San Antonio Breast Cancer Symposium.

- *At a Phase 2 dose of 450 mg, oral once-daily (qd) seviteronel has demonstrated clinical activity and a generally well-tolerated safety profile in women with estrogen receptor-positive (ER+) or triple-negative breast cancer (TNBC)*
- *Phase 2 enrollment is ongoing for Phase 2 breast and castration-resistant prostate cancer (CRPC) trials of seviteronel*
- *Additional Phase 2 breast and prostate cancer program updates will be presented in 2017*

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RESEARCH TRIANGLE PARK, N.C.--(BUSINESS WIRE)--Innocrin Pharmaceuticals, Inc., a clinical-stage company focused on the development of the oral, selective CYP17-lyase/androgen receptor (AR) inhibitor, seviteronel, announced today that clinical results from the ongoing CLARITY (**C**YP17 **L**yase and **A**ndrogen **R**eceptor **I**nhibitor **T**reatment with Seviteronel) study (INO-VT-464-006; NCT02580448) were presented at the 39th annual San Antonio Breast Cancer Symposium (SABCS). The presentation, by Ayca Gucalp, MD, lead author and medical oncologist at Memorial Sloan Kettering Cancer Center, reported results from women with triple-negative or ER+ breast cancer administered seviteronel at 450mg qd.

Title: Phase 1/2 study of oral seviteronel (VT-464), a dual CYP17-lyase inhibitor and androgen receptor (AR) antagonist, in patients with advanced AR positive triple negative (TNBC) or estrogen receptor (ER) positive breast cancer (BC). (Abstract P2-08-04).

Women with either unresectable locally advanced or metastatic ER+ or triple-negative breast cancer were administered seviteronel at the Phase 2 dose of 450 mg qd. The clinical benefit rate (complete response, partial response or stable disease) for at least 16 weeks (TNBC) or 24 weeks (ER+) was the primary endpoint. Clinical activity, endocrine responses and safety were reported as of 04 October 2016 from the initial 17 women (n=11 ER+ and n=6 TNBC) enrolled as of 7 June 2016. Three of 11 women with ER+ breast cancer and 3 of 6 women with TNBC remained on study without

disease progression for at least 147 to 236 days and 135 to 159, respectively. As of 04 October 2016 data cut, all 3 women with ER+ breast cancer and 2 of the women with TNBC were ongoing. Tolerability appeared acceptable with fatigue, dizziness, nausea and decreased appetite being the most frequent AEs and all were grade 1 or 2 in severity except for one grade 3 AE (dizziness).

- Duration of therapy for women with ER+ breast cancer and with AR+ TNBC supports clinical benefit of seviteronel and has met statistical threshold to continue accrual to Stage 2
- Consistent with seviteronel CYP17 lyase inhibition, circulating androgen and estrogen declines were noted
- Seviteronel was generally well tolerated; the majority of AEs were Grade 1 or 2

“The results from this study are quite promising. The long duration of therapy in these patients who have received prior treatment suggests clinical benefit and activity of seviteronel,” said Tiffany A. Traina, M.D., lead investigator and medical oncologist at Memorial Sloan Kettering Cancer Center. “The combined inhibition of CYP17 lyase and the AR that seviteronel provides, represents a new therapeutic approach for patients with breast cancer whose disease has progressed following treatment with currently available anti-hormonal or chemotherapies.”

Edwina Baskin-Bey, M.D., Innocrin Chief Medical Officer stated, “The early clinical benefit signals of seviteronel monotherapy are encouraging given the spectrum of patients enrolled to date. Women investigated range from treatment-naïve to heavily pre-treated and from low to high disease burden. The combined seviteronel inhibition of both sex-steroid production and AR activity may provide a novel treatment option for advanced breast cancer patients.”

The SABCS presentation was the first of several planned medical conference presentations from the ongoing Phase 2 trials of seviteronel for the treatment of breast and prostate cancers resistant to approved hormonal therapies. Innocrin will present expanded CLARITY study results (Stage 1 plus the ongoing Stage 2) at a major medical conference in 2017. Also in 2017, the Company expects to present results from the Phase 2 trials investigating once-daily seviteronel in men with CRPC that has worsened despite treatment with multiple marketed AR-targeted therapies, including abiraterone and enzalutamide.

About Seviteronel (VT-464) Seviteronel is a once-daily oral therapeutic given without prednisone. Seviteronel selectively inhibits CYP17 lyase, an enzyme needed for the synthesis of androgens and estrogens, and also directly blocks the AR.

It is thought that the AR may stimulate disease progression of breast cancer tumors that no longer are ER+ (e.g., are triple-negative) or are ER+ but have become resistant to ER-directed therapies such as aromatase inhibitors or tamoxifen. Preclinical study results presented at the 2015 San Antonio Breast Cancer Symposium, confirmed that seviteronel, presumably due to its multiple mechanisms of action, blocks the growth of resistant ER+ and AR+ breast cancer cells more potently than enzalutamide.

A growing body of preclinical and clinical evidence shows that seviteronel blocks the growth of deadly CRPC that is resistant to abiraterone (a CYP17 hydroxylase inhibitor) or enzalutamide (an AR antagonist). CRPC disease progression following treatment with abiraterone, enzalutamide or both represents a major unmet medical need due to the widespread and growing use of both agents, as well as the high cross-resistance between these agents (e.g., cancers that are resistant to abiraterone are typically resistant to enzalutamide and *vice versa*).

About Breast Cancer Each year over 230,000 women are diagnosed with breast cancer 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 ER+ BCa, the majority of patients eventually develop resistance. ER+ patients comprise ~75% of all metastatic breast cancer cases, and TNBC accounts for ~15-20%. TNBC has a more aggressive course than ER+ BCa does but both have poor survival rates post-failure of endocrine and/or chemotherapy.

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. Prostate cancer afflicts nearly 240,000 men each year in the US and approximately 36,000 men die due to metastatic CRPC.

About Innocrin Pharmaceuticals, Inc. (www.innocrinpharma.com) Innocrin discovers and develops novel oral inhibitors of CYP17 lyase and the AR. Innocrin wholly owns the patents that protect seviteronel and structurally related classes of CYP17 lyase-selective inhibitors. CYP17 lyase inhibitors may have high commercial potential for the

treatment of a wide array of cancers including ovarian, liver, bladder, and head and neck. In addition, Innocrin has plans to develop CYP17 lyase inhibitors for the treatment of non-oncologic syndromes that are due to hormone excess, including endometriosis, polycystic ovary syndrome and congenital adrenal hyperplasia. Innocrin's investors include the Novartis Venture Fund, Eshelman Ventures, Lilly Ventures, Hatteras Venture Partners, Intersouth Partners, Lurie Holdings, and Astellas Venture Management.

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