January 15, 2020 Acurx Pharmaceuticals

Acurx Receives USAN Approval of Non-proprietary Name Ibezapolstat for Its Phase 2 Novel, First-in-Class Antibiotic for C. difficile Infection

- Ibezapolstat is first of a new class of antibiotics, DNA Pol IIIC inhibitors, to begin patient enrollment in a Phase 2 clinical trial in 1Q2020 as an oral treatment for C. difficile infection

- C. difficile bacteria remain on CDC Urgent Threat list, highlighting need for new antibiotics

WHITE PLAINS, N.Y., Jan. 15, 2020 /PRNewswire/ -- Acurx Pharmaceuticals, LLC ("Acurx" or the "Company"), a privately held, clinical stage biopharmaceutical company developing new antibiotics for difficult-to-treat bacterial infections, announced today that the United States Adopted Names Council (USAN) of the American Medical Association has approved the use of *ibezapolstat* (pronounced: eye-be-za-pol-stat) for ACX-362E, the Company's lead antibiotic product candidate in clinical development for the oral treatment of patients with *C. difficile* Infection (CDI).

The U.S. Center for Diseases Control issued its 2019 update on antimicrobial resistance https://www.cdc.gov/drugresistance/pdf/threats-report/2019-ar-threats-report-508.pdf and reaffirmed that *C. difficile* Infection remains an URGENT threat causing at least 12,800 deaths in 2017, highlighting the need for new antibiotics, particularly with a novel mechanism of action. It further reported that more than 2.8 million antibiotic-resistant infections occur in the U.S. each year and more than 35,000 people die as a result, nearly twice as many annual deaths than previously reported by CDC in 2013. These deaths are attributed to antimicrobial-resistant pathogens including *Enterococcus* (including vancomycin-resistant strains or VRE), *Staphylococcus* (including methicillin-resistant strains or MRSA), and *Streptococcus* (including antibiotic-resistant strains) which are the targets of Company's second antibiotic candidate currently in preclinical development.

Having successfully completed the first-in-human Phase 1 trial for this new class of antibiotics which has the novel mechanism of action of blocking DNA synthesis through inhibition of the enzyme (DNA pol IIIC) in Gram-positive bacterial cells, the Company is preparing to begin enrollment of a Phase 2 clinical trial in 1Q2020. Results from the Phase 1 trial, a "Randomized, Double-blind, Placebo-controlled, Single and Multiple Ascending Dose Phase 1 Study to Determine the Safety, Pharmacokinetics, Food, and Fecal Microbiome Effects of ACX-362E [ibezapolstat] Administered Orally to Healthy Subjects," demonstrated excellent tolerability of ibezapolstat along with fecal concentrations comparable to or better than those observed with precedent products that have advanced to demonstrate clinical success.

The Phase 1 clinical trial had a secondary endpoint to determine the fecal microbiome effects of ibezapolstat compared to those of the standard of care, oral vancomycin. Microbiome analysis demonstrated that ibezapolstat had a significantly more favorable effect than oral vancomycin on the microbiome due to less disruption of the favorable microbiota in the GI tract. This microbiome comparison was ground-breaking as it had not been done in prior CDI Phase 1 clinical trials in the U.S. Data from the Phase 1 trial were presented by Dr. Kevin Garey, Professor and Chair, University of Houston College of Pharmacy and the Principal Investigator for the microbiome aspects of the trial at **ID**Week™ in October 2019. Additionally, results from two *in vitro* studies of ibezapolstat against contemporary isolates of *C. difficile* were presented by scientists in poster presentations at the joint ASM/ESCMID Conference on Drug Development in September 2019. These three presentations are available on the Company's website at www.acurxpharma.com.

Robert DeLuccia, Co-Founder & Managing Partner of Acurx stated: "Approval of the ibezapolstat name is a significant milestone along the new drug development pathway marking its later-stage of development and establishing the chemical nomenclature of an entirely new class of antimicrobials by our Company."

About USAN. The United States Adopted Names (USAN) Council of the American Medical Association is responsible for selecting and approving nonproprietary (generic) drug names. The USAN Council establishes logical nomenclature classifications based on pharmacological and/or chemical relationships. In addition to one member-at-large and a Food and Drug Administration (FDA) liaison, the council consists of one representative from each of the following: The AMA, United States Pharmacopeia (USP) and the American Pharmacists Association (APhA).

About the Phase 1 Clinical Trial. ACX-362E, has successfully completed a 62-subject, double-blind, placebo- controlled, multiple-ascending dose Phase 1 clinical trial of ACX-362E as an oral treatment in healthy volunteers. The Phase 1 clinical trial was first-in-man for a new class of antibiotics which work by inhibiting DNA synthesis in certain bacterial cells (pol IIIC inhibitors). Pol IIIC is required for DNA replication of many Gram-positive pathogens, including *Clostridioides* as well as *Enterococcus*, *Staphylococcus*, and *Streptococcus*. Safety information was analyzed through assessment of adverse events and other standard safety measures, while concentrations of ACX-362E were determined in both the blood and the feces, the latter being the critical site of drug delivery for treating CDI. For the microbiome analysis, daily stool samples from subjects in the multiple-ascending arm (MAD) were collected for microbiome analysis. DNA was extracted from stool and sent for shotgun metagenomic sequencing to assess diversity changes in the microbiome. In addition, DNA samples were tested by quantitative polymerase chain reaction (qPCR) to test for quantitative changes in relevant host microbiota.

For the multiple-ascending dose studies, subjects received either ACX-362E 300 mg and 450 mg given twice daily. There were 6 subjects at each dose range compared to six patients that received vancomycin 125 mg given four times daily. All treatments were given for 10 days. Results of the metagenomic sequencing demonstrated a unique microbiome profile for subjects given either dose of ACX-362E compared to the microbiome of subjects that received vancomycin. In qPCR analysis, significantly decreased taxa of Bacteroides, Firmacutes, Prevotella, and two Clostridia species (*C. leptum* and *C. coccoides*) were observed.

About ibezapolstat, FDA QIDP and Fast Track Designation. In June 2018, FDA granted Qualified Infectious Disease Product (QIDP) designation to ibezapolstat as an oral treatment for patients with CDI. In addition, in January 2019, FDA granted Fast Track designation to ibezapolstat for the oral treatment for patients with CDI.

FDA Fast Track Designation is a process designed to facilitate the development and expedite the regulatory pathway of new drugs to treat serious or life-threatening conditions and that fill a high unmet medical need. Ibezapolstat is a novel, first-in-class, orally administered antibacterial. It is the first of a novel class of DNA polymerase IIIC inhibitors under development by Acurx to treat bacterial infections. Acurx acquired ibezapolstat from GLSynthesis, Inc. in February 2018.

FDA's QIDP Designation provides that ibezapolstat will be eligible to benefit from certain incentives for the development of new antibiotics provided under the Generating Antibiotic Incentives Now Act (the GAIN Act). These incentives include Priority Review and eligibility for Fast Track status, the latter of which Acurx has already applied for and been granted by FDA. Further, if ultimately approved by the FDA, ibezapolstat is eligible for an additional five-year extension of Hatch-Waxman marketing exclusivity. Ibezapolstat is being developed as a targeted, narrow spectrum oral antibiotic for the treatment of patients with CDI. Acurx is planning to advance ibezapolstat into a Phase 2 clinical trial in first quarter 2020. The CDC (Centers for Disease Control & Prevention) has designated *Clostridium difficile* bacteria as an urgent threat highlighting the need for new antibiotics to treat CDI.

About *Clostridioides Difficile* Infection (CDI). The CDC has reported that there are nearly 500,000 patients per year treated for CDI in the U.S. alone, with a recurrence rate approximated at 20% to 30%, with limited antibiotics available to treat patients with CDI. CDI is also prevalent in Europe, Japan and Canada, which are countries where the Company has patent protection and anticipates further clinical development and commercialization.

About DNA polymerase IIIC (pol IIIC). Working in scientific collaboration with WuXi AppTec, Acurx has identified additional potential therapeutic candidates to add to its pipeline of DNA polymerase IIIC inhibitors. Nonclinical research has established the mechanism of action of ibezapolstat as the selective inhibition of the enzyme DNA polymerase IIIC (pol IIIC), which is required for bacterial replication and pathogenesis. This enzyme is found only in certain Gram-positive bacteria, including *C. difficile* as well as the pathogens *Enterococcus* (including vancomycin- resistant strains or VRE), *Staphylococcus* (including methicillin-resistant strains or MRSA), and *Streptococcus* (including antibiotic-resistant strains). Accordingly, chemically related molecules with the same mechanism of action as ibezapolstat have the potential to treat a variety of serious systemic Gram-positive infectious diseases.

About Acurx Pharmaceuticals, LLC. Acurx Pharmaceuticals is a privately held clinical stage biopharmaceutical company focused on developing new antibiotics for difficult to treat infections. Acurx's approach is to develop antibiotic candidates that could potentially block an entirely new molecular target, DNA polymerase IIIC (pol IIIC) and its R&D pipeline includes early stage antibiotic candidates that target other Gram-positive bacteria that are active parenterally, and potentially orally, including Methicillin-Resistant *Staphylococcus aureus* (MRSA), Vancomycin- Resistant Enterococcus (VRE) and Penicillin-Resistant *Streptococcus pneumoniae* (PRSP).
For more information, please visit our website at[www.acurxpharma.com.](http://www.acurxpharma.com)

Any statements in this press release about our future expectations, plans and prospects, including statements regarding our strategy, future operations, prospects, plans and objectives, and other statements containing the words "believes," "anticipates," "plans," "expects," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: whether ibezapolstat will benefit from the QIDP designation; whether ibezapolstat will advance through the clinical trial process on a timely basis; whether the results of the clinical trials of ibezapolstat will warrant the submission of applications for marketing approval, and if so, whether ibezapolstat will receive approval from the United States Food and Drug Administration or equivalent foreign regulatory agencies where approval is sought; whether, if ibezapolstat obtains approval, it will be successfully distributed and marketed; and other factors. In addition, the forward-looking statements included in this press release represent our views as of January 15, 2020. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward- looking statements at some point in the future, we specifically disclaim any obligation to do so.

Investor Contact: Acurx Pharmaceuticals, LLC
David P. Luci Co-Founder & Managing Partner; 917-533-1469;
davidluci@acurxpharma.com

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