November 5, 2020 Acurx Pharmaceuticals

# Acurx Announces All Patients Demonstrated Clinical and Sustained Cure in Ph2A Open-Label Trial of Ibezapolstat for Treatment of C. difficile Infection (CDI)

**- Ibezapolstat is the first of an entirely new class of antibiotics with a novel mechanism of action, a DNA polymerase IIIC inhibitor, to enter clinical efficacy trials**

**- 10 of 10 patients enrolled in the Ph2A trial met the study's primary and secondary efficacy endpoints**

**- No C. difficile was detected in any of the 65 fecal samples tested by Day 3 of treatment and thereafter**

**- Compelling evidence of efficacy allows early termination of Segment 2A and advancement to Segment 2B**

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

**- Ibezapolstat is FDA QIDP and Fast Track Designated for priority review**

WHITE PLAINS, N.Y., Nov. 5, 2020 /PRNewswire/ -- Acurx Pharmaceuticals, LLC ("Acurx" or the "Company"), a privately held, clinical stage biopharmaceutical company developing an entirely new class of antibiotics for difficult-to-treat bacterial infections, announced that all 10 patients (100%) with mild-to-moderate CDI enrolled in this Ph2A open-label clinical trial met the study's primary and secondary efficacy endpoints following treatment with orally administered ibezapolstat given 450 mg twice daily for 10 days. FDA has granted Qualified Infectious Disease Product (QIDP) designation and Fast-Track status to ibezapolstat for patients with CDI.

In this Phase 2 clinical trial, Segment 2A was designed to enroll up to 20 patients with a data review planned by a Trial Oversight Committee after 10 patients completed the trial. All 10 patients enrolled in the trial met the study's primary and secondary efficacy endpoints of Clinical Cure at end of treatment and Sustained Clinical Cure of no recurrence of CDI at the 28-day follow-up visit. Ibezapolstat was well-tolerated, with no serious adverse events (SAEs) reported in the trial. Based on these successful treatment results, and in consultation with the Company's medical advisors, the Company has terminated enrollment in Segment 2A early and will advance to Segment 2B. These results also represent the first-ever clinical validation of DNA polymerase IIIC as a therapeutically relevant antibacterial target.

Dr. Kevin Garey, Professor and Chair, University of Houston College of Pharmacy and the Principal Investigator for the microbiome aspects of the trial stated, "Data from my laboratory confirm that ibezapolstat eradicated C. difficile in all fecal samples tested by Day 3 of treatment in this patient population with mild or moderate CDI. Our ongoing work will characterize the effects of ibezapolstat on the intestinal microbiome in these CDI patients. A strength of this development program will be the ability to compare the microbiome results in CDI patients to our prior favorable effects relative to vancomycin previously demonstrated in the Phase 1 healthy volunteer trial."

Stuart Johnson, MD, Professor of Medicine at Loyola University, a CDI expert and an Acurx Scientific Advisory Board (SAB) member, noted, "After reviewing Segment 2A , the SAB is encouraged by the promising results and fully support early termination and advancement to the Ph2B Segment with the next enrolled patient. The SAB looks forward to successful results from Segment 2B that could pave the way for an important new antibiotic class for treatment of CDI which remains an area of clear medical need."

These data will be presented at the 8th Annual International *C. diff*. Virtual Conference and Virtual Health Expo on November 14, 2020 **https://cdiff2020.com** which coincides with the US Centers for Disease Control and Prevention (CDC) declaration of November as *Clostridioides difficile* Awareness Month.

Robert J. DeLuccia, Co-Founder & Managing Partner of Acurx, stated, "We are very excited by these excellent results allowing early termination of our Phase 2A Segment at 10 patients. We are looking forward to starting our Phase 2B Segment early next year with expectation for completion by the end of next year". He further stated, "since ibezapolstat is the first DNA polymerase IIIC inhibitor to advance into clinical trials, this achieves the first human validation of our bacterial target and will enable further development of our pipeline of novel oral and I.V. antibiotics with the same bacterial target and mechanism of action. Our new compounds are in pre-clinical development to treat other Gram-positive life-threatening infections in skin/skin structure, community acquired pneumonia, bone & joint and bacteremia. The spectrum of activity of Acurx's DNA pol IIIC inhibitors includes pathogens resistant to currently available antibiotics and classified as priority pathogens by the WHO, CDC and FDA, all of whom emphasize the need for new classes of antibiotics to prepare for the next global infectious disease pandemic, antimicrobial resistance."

**About the Phase 2 Clinical Trial.** In Segment 2A of this trial, 10 subjects with diarrhea caused by *C. difficile* were treated with ibezapolstat 450 mg orally for 10 days and evaluated for clinical cure. All cured subjects were followed for sustained clinical cure at 28 ± 2 days. In Segment 2B, approximately 64 additional subjects with CDI will be enrolled and randomized in a 1:1 ratio to either ibezapolstat 450 mg every 12 hours or vancomycin 125 mg orally every 6 hours for 10 days and will be followed for 28 ± 2 days for recurrence. The two treatments will be identical in appearance, dosing times, and number of capsules administered to maintain the blind. Subjects in both segments will be evaluated for clinical and sustained clinical cure, safety, and tolerability. All subjects in both segments will have stool samples tested for microbiome profiles. Additional information about the trial, including eligibility criteria can be found at: www.clinicaltrials.gov (Study identifier: NCT04247542).

**About *Clostridioides Difficile* Infection (CDI).** *Clostridioides* (formerly *Clostridium) difficile,* also known as *C. difficile* or *C. diff,* is one of the most common

causes of health care-associated infections in U.S. hospitals (Lessa, et al, 2015, New England Journal of Medicine). Recent estimates suggest *C. difficile* approaches 500,000 infections annually in the United States and is associated with approximately 20,000 deaths. (Guh, 2020, New England Journal of Medicine). Based on internal estimates including a recurrence rate of approximately 20%, we believe the annual incidence in the U.S. approaches 600,000.

**About the C Diff Foundation:** The Company recognizes the month of November as*C. Difficile* Awareness Month as designated by the US Centers for Disease Control and Prevention (CDC) and supports the work of the C Diff Foundation in educating and advocating for the Prevention, Treatments, Clinical Trials, and Environmental Safety of *Clostridioides difficile (C.difficile*) Infections worldwide. https://cdifffoundation.org/. The C Diff Foundation recently announced the release of **C diff and You** app, available from the Apple Store (apple.com) and Google Store (play.google.com). Developed with patients, family members, and caregivers in mind, the app provides information about *C. difficile* infection prevention, treatments, clinical trials, support, guidelines, environmental safety and nutrition.

**The U.S. Center for Diseases Control 2019 Update on Antimicrobial Resistance.** https://www.cdc.gov/drugresistance/pdf/threats-report/2019-ar-threats-report-508.pdf CDC 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 antibiotic pipeline currently in preclinical development and also eligible for QIDP and FDA-Fast-Track-Designation for priority review.

**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'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.

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 new class of DNA polymerase IIIC inhibitors in clinical development by Acurx to treat bacterial infections.

**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 pol IIIC inhibitors. Nonclinical research has established the mechanism of action of ibezapolstat as the selective inhibition of the enzyme DNA polpol 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 target the DNA polymerase IIIC enzyme and its R&D pipeline includes early stage antibiotic candidates that target other Gram-positive bacteria, 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 November 5, 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.

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