New antibiotic shows promising results in clinical trials

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QPX9003, a novel antibiotic developed by Monash University researchers targeting antibiotic-resistant "superbugs" has achieved an important milestone in its clinical development, with the initial Phase 1

studies of the drug delivering very promising safety and tolerability results.

Data presented at IDWeek 2022 in Washington DC in October included results from the Phase 1 placebo-controlled randomized single ascending doses (SAD) and multiple ascending doses (MAD) of QPX9003 in healthy adults.

Data show that the drug was well tolerated in single dose escalation up to 400 mg and multiple doses up to 600 mg per day for 14 days. No nephrotoxicity or other significant adverse events were observed and, importantly, no subjects discontinued the trial. Based on its pharmacokinetic and safety profile, and results in nonclinical models of infection, it is anticipated that QPX9003 can be safely dosed at levels that would be sufficient to achieve clinical efficacy and warrants further clinical development.

QPX9003, is an intravenously administered synthetic lipopeptide for treating gram-negative infections including those due to "superbugs" that are resistant to many classes of antibiotics. QPX9003 was discovered at the Monash Institute of Pharmaceutical Sciences (MIPS) and the Monash Biomedicine Discovery Institute (BDI), The team of Monash researchers includes Professor Jian Li, Associate Professor Tony Velkov, Dr. Kade Roberts, Professor Roger Nation and Professor Philip Thompson.

"These Phase 1 results are very inspiring as the currently used treatments, polymyxin B and colistin, display significant toxicity issues in the clinic that have severely limited their use," said Professor Li who heads up the Monash antibiotic drug discovery program. "These results showing the safety and tolerability for QPX9003 at clinically relevant doses is critical and demonstrates that QPX9003 has the potential to be administered at significantly higher doses than polymyxin B and colistin

without adverse effects," he added.

The Phase 1 study was conducted by the US-based biopharmaceutical company Qpex Biopharma Inc, in partnership with the U.S. Department of Health and Human Services, Biomedical Advanced Research and Development Authority (BARDA). Qpex has worked in collaboration with Monash researchers to discover new antibiotics and licensed the drug from Monash to support the development of QPX9003. A paper led by Monash researchers detailing the research that led to QPX9003 becoming a clinical candidate was published earlier this year in *Nature Communications*.

Gram-negative bacteria can cause serious infections, including pneumonia, bloodstream infections, urinary tract infections, peritonitis and meningitis. The World Health Organization has highlighted that new antibiotics are urgently needed to treat bacterial "superbugs," which have the potential to kill 10 million people per year by 2050—more than any other type of disease. No no new lipopeptide antibiotics have been approved against Gram-negative pathogens since polymyxin B and colistin became available in the late 1950s.

"It is exciting to see the progress of QPX9003 advancing as part of our portfolio of clinical stage investigational antibiotic programs," said Michael Dudley, PharmD, president and chief executive officer of Qpex. "QPX9003 has the potential to provide a new treatment option for patients with drug-resistant infections for which there are limited treatment options. We look forward to discussions with regulators on the next steps toward studies in patients," he added.

QPX9003 has already been granted a Qualified Infectious Disease Product (QIDP) designation by the FDA, providing drug candidates with this designation potential incentives such as regulatory exclusivity extensions, and eligibility for fast-track designation.

Provided by Monash University