# HOOKIPA Announces Positive Phase 2 Interim Safety, Immunogenicity, and Efficacy Data for its Cytomegalovirus Vaccine Candidate HB-101

November 30, 2020

- Early interim data show Cytomegalovirus (CMV)-negative kidney transplant recipients vaccinated with three doses of HB-101 had reduced incidence of CMV viremia, reduced antiviral use and no CMV disease
- Observed CMV-neutralizing antibody responses and tolerability profile are consistent with previous interim results
- CMV can cause severe complications in kidney transplant recipients including organ rejection and death

NEW YORK and VIENNA, Austria, Nov. 30, 2020 (GLOBE NEWSWIRE) -- HOOKIPA Pharma Inc. (NASDAQ: HOOK, 'HOOKIPA'), a company developing a new class of immunotherapeutics based on its proprietary arenavirus platform, today announced positive interim efficacy results, as well as additional safety and immunogenicity data, for its prophylactic Cytomegalovirus (CMV) vaccine candidate HB-101. HB-101, a non-replicating arenavirus vaccine, is being investigated in a double-blind Phase 2 clinical trial (NCT03629080) to assess safety, immunogenicity and efficacy in individuals receiving a kidney transplant from a living donor. HOOKIPA will host a conference call and live audio webcast today at 8:30am EST.

CMV infection is one of the most common complications affecting solid organ transplant recipients and can result in serious conditions like hepatitis and pneumonia, as well as increase the likelihood of transplant rejection and graft-versus-host disease. Antiviral therapies are used to help control disease, but they are limited by toxicity and the emergence of viral resistance.[1]

The interim efficacy analysis includes data from 41 participants as of the cut-off date; 8 were vaccinated with three doses of HB-101 pre-transplant, 19 were vaccinated with two doses and 14 received placebo.

Compared to placebo, participants vaccinated with three HB-101 doses had:

- a 48 percent reduction in CMV viremia (presence of CMV DNA in the blood);
- a 42 percent reduction in the use of antiviral therapy; and
- no CMV disease (compared to 2 out of 14 cases in the placebo group)

Response to a two-dose schedule did not show an improvement compared to placebo, which is consistent with the low levels of CMV-neutralizing antibody in the two-dose group as well as with the T cell data reported in June 2020 for the two-dose group.

"While these interim data are from a small group of patients, they offer early insight into the potential of a three-dose schedule of HB-101 to help protect kidney transplant recipients against CMV disease," said Joern Aldag, Chief Executive Officer of HOOKIPA. "If these trends continue, HB-101 may be a promising first-in-class vaccine candidate to help address an unmet need in this vulnerable patient population. We are excited to explore a path to a Phase 3 CMV vaccine program, as well as to see how these early efficacy signals with our non-replicating arenavirus technology may translate to our replicating technology in oncology."

The interim immunogenicity analysis also included CMV-neutralizing antibody data assessed from 33 individuals, a subset of the 41 included in the efficacy analysis. 21 participants were vaccinated with HB-101 and 12 received placebo. In line with previous interim data, 100 percent of the participants who received three doses of HB-101 mounted CMV-neutralizing antibodies.

Safety and tolerability were evaluated in 69 participants who were enrolled in the trial by the cut-off date. HB-101 was generally well tolerated with a low incidence of side effects, which were mostly mild to moderate. Specifically, 17 percent of participants across the combined HB-101 and placebo groups showed side effects related to vaccine administration. Three cases of human leukocyte antigen (HLA)-sensitization have been reported, two as serious adverse events. HLA-sensitization can cause the recipient to identify the donor kidney as foreign and may require a new donor to be identified. HLA-sensitization is a known complication of dialysis patients waiting for kidney transplantation, affecting an estimated 4% of this patient population[2].

"CMV is a key threat to the health and overall prognosis of kidney transplant recipients," said Paul Griffiths, MD DSc FRCPath, Professor of Virology, Institute of Immunity & Transplantation at University College London. "While antivirals are an important tool, we need better interventions to reduce the risk of CMV disease in this vulnerable population. These preliminary data highlight the potential for a new standard of care in the management of kidney transplant recipients."

**About the trial**
This double-blind Phase 2 clinical trial is designed to assess safety, immunogenicity and efficacy in individuals receiving a kidney transplant from a live donor to measure the decrease of post-transplant viremia in the absence and presence of antivirals. Among CMV-negative participants, individuals are blinded and randomized 2:1 to receive either HB-101 or placebo. Depending on the transplantation time schedule, participants are vaccinated with either two or three doses prior to transplantation. Participants receive either pre-emptive or prophylactic antiviral therapy post-transplant and are followed for a 12-month observation period. Among CMV-positive participants, individuals receive either two or three doses of HB-101, pre-emptive or prophylactic antiviral therapy, and are followed for a 12-month observation period.

**Conference call**

HOOKIPA will host a conference call and live audio webcast today at 8:30am EST to discuss the CMV data. To access the conference call, please dial +1 877 870 9135 (from the US) or +44 2071 928338 (international) and refer to conference ID 4469307. The webcast and the presentation will be available within the Investors & Media section of HOOKIPA's website at https://ir.hookipapharma.com/events. An archived replay will be accessible for 30 days following the event.

**About Cytomegalovirus**

Cytomegalovirus, or CMV, is a type of herpesvirus that infects the majority of people over the course of their lifetime. The U.S. Centers for Disease Control and Prevention estimates that more than half of adults have been infected with CMV by age 40.[3] The majority of CMV infections are not serious, and the virus can lay dormant in the body for years. However, CMV infection poses a considerable risk to infants in utero, as well as immune-compromised individuals, such as solid organ transplant recipients. There are currently more than 90,000 people on the kidney transplant waiting list in the United States.[4]

CMV is one of the most common complications affecting solid organ transplant recipients.[5] Despite active monitoring and antiviral therapy, CMV infection occurs in 20-70 percent of transplant recipients the first year after transplant.[6] Within this population, CMV disease most commonly causes fever, low white blood cell count (leukopenia), low platelet levels (thrombocytopenia) and elevated liver function. It also can cause more serious conditions like hepatitis and pneumonia, which often require hospitalization, as well as increase the likelihood of transplant rejection and graft-versus-host disease.[7]

**About HOOKIPA**

HOOKIPA Pharma Inc. (NASDAQ: HOOK) is a clinical stage biopharmaceutical company developing a new class of immunotherapeutics based on its proprietary arenavirus platform that reprograms the body's immune system. HOOKIPA's proprietary arenavirus-based technologies, non-replicating (VaxWave®) and replicating (TheraT®), induce robust antigen-specific CD8+ T cells and pathogen-neutralizing antibodies. HOOKIPA's viral vectors target antigen presenting cells in vivo to activate the immune system. Both technologies enable repeat administration to augment and refresh immune responses. As a monotherapy, our replicating arenavirus technology has the potential to induce CD8+ T cell response levels previously not achieved by other immuno-therapy approaches.

HOOKIPA's non-replicating prophylactic cytomegalovirus (CMV) vaccine candidate is currently in a Phase 2 clinical trial for patients awaiting kidney transplantation. To expand its infectious disease portfolio, HOOKIPA entered into a collaboration and licensing agreement with Gilead Sciences, Inc. to research arenavirus-based functional cures for HIV and chronic Hepatitis B infections.

In addition, HOOKIPA is building a proprietary immuno-oncology pipeline by targeting virally mediated cancer antigens, self-antigens and next-generation antigens. The lead replicating arenavirus oncology product candidates, HB-201 and HB-202, are in development for the treatment of Human Papilloma Virus 16-positive cancers in a Phase 1/2 clinical trial.
Find out more about HOOKIPA online at www.hookipapharma.com.

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[1] Gilbert C and Boivin G. Human Cytomegalovirus Resistance to Antiviral Drugs. *Antimicrobial Agents and Chemotherapy.* 2005; 49(3):873-883. Available at: https://aac.asm.org/content/aac/49/3/873.full.pdf

[2] Nephrol Dial Transplant (2013) 28: 2908–2918

[3] Centers for Disease Control and Prevention. About Cytomegalovirus (CMV). Available at: https://www.cdc.gov/cmv/overview.html

[4] Organ Procurement and Transplantation Network. Kidney transplant waiting list. Available at: https://optn.transplant.hrsa.gov/data/

[5] Kotton CN et al. The Third International Consensus Guidelines on Management of Cytomegalovirus in Solid-organ Transplantation. *Transplantation.* 2019; 102: 900-931. Available at: https://journals.lww.com/transplantjournal/Fulltext/2018/06000/The_Third_International_Consensus_Guidelines_on.13.aspx

[6] Cui X, CM Snapper. "Development of novel vaccines against human cytomegalovirus." *Human Vaccines & Immunotherapeutics.* 2019, vol 15, no 11, 2673-2683. Available at: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6930071/pdf/khvi-15-11-1593729.pdf

[7] Kotton CN et al. The Third International Consensus Guidelines on Management of Cytomegalovirus in Solid-organ Transplantation. *Transplantation.* 2019; 102: 900-931. Available at: https://journals.lww.com/transplantjournal/Fulltext/2018/06000/The_Third_International_Consensus_Guidelines_on.13.aspx

**HOOKIPA Forward Looking Statements**

Certain statements set forth in this press release constitute "forward-looking" statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. Forward-looking statements can be identified by terms such as "believes," "expects," "plans," "potential," "would" or similar expressions and the negative of those terms. Such forward-looking statements involve substantial risks and uncertainties that could cause HOOKIPA's research and clinical development programs, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the uncertainties inherent in the drug development process, including HOOKIPA's programs' early stage of development, the process of designing and conducting preclinical and clinical trials, the regulatory approval processes, the timing of regulatory filings, the challenges associated with manufacturing drug products, HOOKIPA's ability to successfully establish, protect and defend its intellectual property, risks relating to business interruptions resulting from the coronavirus (COVID-19) disease outbreak or similar public health crises, the impact of COVID-19 on the enrollment of patients and timing of clinical results for HB-101 and other programs, and other matters that could affect the sufficiency of existing cash to fund operations and HOOKIPA's ability to achieve the milestones under the agreement with Gilead. HOOKIPA undertakes no obligation to update or revise any forward-looking statements. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of the company in general, see HOOKIPA's quarterly report on Form 10-Q for the quarter ended September 30, 2020 which is available on the Security and Exchange Commission's website at www.sec.gov and HOOKIPA's website at www.hookipapharma.com.

Investors and others should note that we announce material financial information to our investors using our investor relations website (https://ir.hookipapharma.com/), SEC filings, press releases, public conference calls and webcasts. We use these channels, as well as social media, to communicate with our members and the public about our company, our services and other issues. It is possible that the information we post on social media could be deemed to be material information. Therefore, we encourage investors, the media, and others interested in our company to review the

information we post on the U.S. social media channels listed on our investor relations website.

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