Vedanta Biosciences Announces Initiation of Phase 1a/1b Trial for New Drug Class of Rationally-Defined Bacterial Consortia Derived from the Human Microbiome

VE303 is first known rationally-defined bacterial consortium in powder form to enter the clinic and has received Orphan Drug Designation from the U.S. FDA for C.* difficile *indication

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Vedanta Biosciences, an affiliate of PureTech Health (LSE: PRTC) developing a new category of therapies for immune-mediated and infectious diseases based on rationally defined consortium of human microbiome-derived bacteria, today announced the initiation of a Phase 1a/1b, first-in-human, clinical trial of VE303, the Company's lead, orally-administered microbiome therapeutic product candidate for recurrent Clostridium *difficile* infection (rCDI). VE303 is the first known investigational drug consisting of a rationally-defined bacterial consortium in powder form to enter the clinic. Vedanta also today announced that VE303 has been granted Orphan Drug Designation by the United States Food and Drug Administration (FDA).

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"The initiation of this study marks an important milestone for both Vedanta Biosciences and the microbiome field, as we've been able to advance a new class of drugs to the clinic," said Bernat Olle, Ph.D., Chief Executive Officer of Vedanta Biosciences. "First-generation approaches in the microbiome field have relied on uncharacterized fecal material or spore fractions of fecal material, which are inherently inconsistent procedures. Products with a rationally-defined composition are the next logical step in the evolution of the microbiome field. We believe VE303 is the first rationally-defined bacterial consortium in powder form to advance into the clinic in any indication, and we plan to rapidly advance two additional programs into clinical development in 2018."

VE303 is an orally-administered investigational microbiome therapeutic consisting of live bacteria designed to restore colonization resistance against gut pathogens, including C. *difficile*, following recurrence. The Phase 1a/1b, dose-escalating study will assess the safety, tolerability, and colonization of VE303 in healthy volunteers, and will enroll approximately 30 subjects. The primary outcome of the study is the evaluation of the safety and tolerability of VE303, and secondary outcomes include the kinetics of intestinal colonization by the bacteria in VE303. The study is expected to be completed in the first half of 2018. Pending the results of this study, Vedanta plans to initiate a Phase 2 trial in rCDI in 2018.

VE303 has also been granted Orphan Drug Designation by the United States Food and Drug Administration (FDA). Orphan Drug Designation is a status given to drugs and biologics that are intended for the safe and effective treatment, diagnosis or prevention of rare diseases or disorders that affect fewer than 200,000 people in the U.S. The Centers for Disease Control and Prevention (CDC) considers C. *difficile* infections one of the most urgent bacterial threats. *C. difficile* infections account for 15,000 deaths each year in the U.S. alone. Existing interventions for C. *difficile* infections include antibiotics, which have the undesirable side effect of damaging the gut microbiome, leaving patients vulnerable to re-infection and building resistance to antibiotics. Microbiome-based therapies offer an alternative to antibiotics that could address both of these problems at once.

About VE303

VE303 is an orally-administered investigational microbiome therapeutic. It is produced from pure, clonal cell banks, which yield a standardized drug product in powder form and bypass the need to rely on direct sourcing of fecal donor material of inconsistent composition. It consists of a defined consortium of live bacteria designed to restore colonization resistance against gut pathogens, including C. *difficile*. VE303 was generated using Vedanta's proprietary discovery platform, which leverages what is believed to be the largest collection of human microbiome-associated bacterial strains, and is manufactured in-house at Vedanta's state-of-the-art, cGMP-compliant facilities.

In November 2017, the VE303 program received a grant of up to $5.4 million from CARB-X (Combating Antibiotic Resistant Bacteria Biopharmaceutical Accelerator), a global public-private partnership focused on funding the development of promising new antibacterial products and rapid diagnostics. This was CARB-X's first award announced for a microbiome project and also the first for a project targeting C. *difficile*.

About Vedanta Biosciences

Vedanta Biosciences is pioneering development of a new category of therapies for immune-mediated and infectious diseases based on rationally designed consortia of bacteria derived from the human microbiome. An affiliate of PureTech Health (PureTech Health plc, PRTC.L), Vedanta's founding team includes a group of world-renowned experts in immunology and microbiology. Vedanta Biosciences is a leader in the microbiome field with capabilities and deep expertise to discover, develop and manufacture drugs based on live bacterial consortia. The Company's facilities include integrated manufacturing operations providing cGMP-compliant manufacturing of rationally-designed bacterial consortia in powder form. Leveraging its proprietary technology platform and the expertise of its team of scientific co-founders, Vedanta Biosciences has isolated and maintains the largest collection of human microbiome-associated bacterial strains and has characterized, in collaborations with leading experts, how the immune system recognizes and responds to these microbes. This pioneering work has led to the identification of human commensal bacteria that induce a range of immune responses – including induction of regulatory T cells, CD8+ T cells, and Th17 cells, among others – as well as the characterization of novel molecular mechanisms of microbial-host communication. These advances have been published in leading peer-reviewed journals including *Science (multiple)*, *Nature (multiple)*, *Cell* and *Nature Immunology*. Vedanta Biosciences has harnessed these biological insights, its proprietary library of microbiome-derived bacterial strains, as well as data from clinical translational collaborations, to generate a pipeline of programs addressing infectious diseases, autoimmune diseases, inflammation and immune-oncology indications.

Vedanta Biosciences' scientific co-founders have pioneered the fields of innate immunity, Th17 and regulatory T cell biology, and include Dr. Ruslan Medzhitov (Professor of Immunobiology at Yale), Dr. Alexander Rudensky (tri-institutional Professor at the Memorial Sloan-Kettering Institute, the Rockefeller University and Cornell University), Dr. Dan Littman (Professor of Molecular Immunology at NYU), Dr. Brett Finlay (Professor at the University of British Columbia) and Dr. Kenya Honda (Professor, Keio University School of Medicine). Vedanta is backed by Seventure, Invesco Asset Management, and Rock Springs Capital and has collaborations with leading institutions including Janssen Biotech, Inc., NYU Langone Health and its Perlmutter Cancer Center, Stanford University School of Medicine, Leiden University Medical Center, University of Tokyo, Keio University, RIKEN, and the University of South Alabama Mitchell Cancer Institute.