Poster #21

TOP-LINE RESULTS FROM VESIGENURTACEL-L (HS-410) IN COMBINATION WITH BCG FROM A RANDOMIZED, BLINDED PHASE 2 TRIAL IN PATIENTS WITH NON-MUSCLE INVASIVE BLADDER CANCER (NMIBC)

Gary Steinberg MD[1], Neal D. Shore MD[2], Lawrence Karsh MD[3], James L. Bailen MD4, Trinity J. Bivalacqua MD PhD5, Karim Chamie MD6, James Cochran MD7, Richard David MD8, Robert Grubb MD?, Wael Harb MD[1°], Jeffrey Holzbeierlein MD[11], Ashish M. Kamat MD[12], Vijay Kasturi MD[13], Edouard J. Trabulsi MD[14], Michael Williams MD[15], Frederick N. Wolk MD8, Michael E. Woods MD[16], Melissa Price PhD[17], Brandon Early MS[17] and Taylor H. Schreiber MD PhD[17]

[1]University of Chicago Medical Center, Chicago IL; [2]Carolina Urologic Research Center, Myrtle Beach, SC; [3]The Urology Center of Colorado, Denver, CO; 4First Urology, Jeffersonville, IN; 5Johns Hopkins University, Baltimore, MD; 6University of California Los Angeles, Los Angeles, CA; 7Urology of North Texas, Dallas, TX; 8Skyline Urology, Torrance, CA; ?Washington University of St. Louis, St. Louis, MO; [1°]Horizon Oncology, Lafayette, IN; [11]Kansas University Medical Center, Westwood, KS; [12]MD Anderson Cancer Center, Houston, TX; [13]University of Massachusetts Memorial Medical Center, Worcester MA; [14]Thomas Jefferson University, Philadelphia, PA; [15]Urology of Virginia, Virginia Beach, VA; [16]University of North Carolina, Chapel Hill, NC; [17]Heat Biologics Inc.

Presented By: Gary David Steinberg, MD

Introduction and Objectives: Vesigenurtacel-L (HS-410) is a vaccine comprised of an allogeneic cell line, selected for high expression from a series of bladder tumor antigens, which has been transfected with gp96-Ig. Cell-secreted gp96-Ig delivers these cell-derived antigens directly to a recipient's own antigen presenting cells, resulting in highly selective activation of CD8+ cytotoxic T cells. Here we present, for the first time, unblinded primary endpoint data (1-year Recurrence-Free Survival (RFS)) from a randomized Phase 2 trial with vesigenurtacel-L in combination with BCG in NMIBC. Trial ID: NCT02010203

Methods: 78 patients with intermediate- (n=5) or high-risk (n=73) NMIBC who are either BCG-naïve or recurrent, with or without carcinoma in situ (CIS), were enrolled 1:1:1 to one of two doses of vesigenurtacel-L (either 10^6 or 10^7 cells/dose) or placebo in combination with 6 weeks of induction BCG, followed by 6 more weeks of vesigenurtacel-L in the induction phase. Maintenance treatment in combination with BCG continued in patients without evidence of disease for 3 courses of 3-weekly treatments at the following timepoints: 3 months, 6 months, 12 months. Concurrently, 16 patients (1 intermediate risk, 15 high-risk) were enrolled in an open-label monotherapy vesigenurtacel-L arm for patients who will not receive BCG. The primary endpoint is 1-year RFS. Secondary efficacy evaluations include recurrence and progression at various timepoints, and analyses of immunologic response in peripheral blood and tumor.

Results: Vesigenurtacel-L treatment was well tolerated with no vaccine-related SAEs; primary AEs were mild, most commonly transient injection site reactions. AE profiles (number and severity of AEs) were similar across the treatment arms indicating that vesigenurtacel-L does not significantly alter the known safety profile of BCG. Composite RFS across all arms (prior to the unblinding event at 1-year) was 84.6%, with a 6-month complete response rate in CIS patients of 87.5%. Vesigenurtacel-L antigen expression showed prominent overlap with patient tumors. Additionally, IHC may define a responder and non-responder phenotype by baseline levels of TIL and PD-L1.

Conclusions: The combination of vesigenurtacel-L and BCG is well-tolerated with preliminary evidence of synergistic effect and immunologic responses that are consistent with vaccine mechanism of action. Vesigenurtacel-L warrants further investigation as a potential treatment for NMIBC.