

March 26, 2014

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (BC-96) VESIGENURTUCEL-L

PRONUNCIATION ves" i jen" ur too' sel - el

THERAPEUTIC CLAIM Cellular immunotherapy for urinary cancer treatment

CHEMICAL DESCRIPTION

The compound consists of a human urothelial cancer cell line, designated UM-UC-3, that is stably transfected with a plasmid eDNA (B45-neo-gp96-Ig-HLA A1). The drug is a whole cell vaccine that has been irradiated to render cell replication incompetent while maintaining biological activity, i.e., expression and secretion of gp96-Ig.

STRUCTURAL FORMULA

The drug consists of UM-UC-3 cells that are transfected to secrete the highly immunogenic protein, gp96-Ig. UM-UC-3 cells express a profile of tumor antigens that are shared by the majority of patients who develop bladder cancer. The transfected protein, gp96-Ig, functions dually as an antigen carrier and adjuvant. Upon injection, UM-UC-3 cells actively secrete gp96-Ig in a complex with the full repertoire of tumor antigens expressed by UM-UC-3 cells. Upon release, gp96-Ig/antigen complexes interact with patient antigen presenting cells via TLR-2, TLR-4 and CD91. Interaction with TLR-2 and TLR-4 provides an adjuvant signal to patient antigen presenting cells, facilitating proper immune activation. CD91 enables endocytosis of gp96-Ig/antigen complexes by activated patient antigen presenting cells. Following endocytosis of gp96-Ig/antigen complexes, patient antigen presenting cells cross-present tumor antigens via MHC I to stimulate a bladder- cancer specific cytotoxic T cell response.

TRADEMARK None as yet

SPONSOR Heat Biologics

CODE DESIGNATIONS HS-410; UM-UC-3-gp96-Ig-HLA-A1

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