AIDS MALIGNANCY CONSORTIUM

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FOR IMMEDIATE RELEASE
9 A.M. EDT, May 31, 2011

TOPICAL AGENT IN AIDS-RELATED KAPOSI SARCOMA TREATMENT: AMC STUDY # 036

ROCKVILLE, MD, MAY 11, 2011: A phase II trial of topical halofuginone (Tempostatin) in AIDS-related Kaposi's sarcoma was conducted on a sample of 23 men. Halofuginone has shown promise as an anti-cancer agent in a variety of laboratory studies. It stops cancer cell growth in several ways, including preventing the formation of new blood vessels (angiogenesis) that can promote spread of cancer cells (metastases) to distant sites. Halofuginone was applied as an ointment directly to Kaposi's sarcoma lesions on the skin. The main purpose of this study was to see if halofuginone could cause the tumors to shrink in people who were on stable HIV medicines (antiretrovirals). A second purpose was to better understand how halofuginone fights cancer by studying the biopsies of Kaposi's sarcoma lesions.

Study volunteers received two tubes of ointment: one tube contained halofuginone and the other tube contained ointment without halofuginone. Volunteers applied halofuginone ointment to the same six Kaposi's sarcoma lesions and ointment without halofuginone to another six Kaposi's sarcoma lesions for the duration of the study. Tumors were measured every 4 weeks for a total of 12 weeks. Neither study volunteers nor AMC investigators knew which tube contained halofuginone ointment and which tube contained ointment without halofuginone. At the end of 12 weeks, for those volunteers whose Kaposi's sarcoma lesions were stable or shrinking while on treatment, there was an option to apply halofuginone ointment to all the Kaposi's sarcoma lesions for an additional 12 weeks.

Treatment was well tolerated. Minor skin reactions were the most common side effects, and there were no significant systemic side effects. None of the 10 volunteers who had blood tests to see if halofuginone was absorbed had detectable levels of the drug in their blood. The response rate to halofuginone ointment was 35% and the response rate to ointment alone was 24%. This difference in Kaposi's sarcoma response between halofuginone ointment and ointment without halofuginone was not statistically significant. This means that the difference in response between the two types of cream was more likely due to chance alone and not because one ointment was more effective than the other in causing Kaposi's sarcoma lesions to shrink.

An important component of this study was the collection of Kaposi's sarcoma biopsy samples. Skin lesions that were treated with halofuginone ointment were more likely to have a decrease in a factor that promotes new blood vessel growth (angiogenesis).

In conclusion, volunteers who applied halofuginone ointment to Kaposi's sarcoma lesions experienced few and modest side effects. The actual shrinkage of Kaposi's sarcoma lesions, however, was not significantly different when compared to an ointment that did not contain halofuginone. One reason for shrinkage of Kaposi's sarcoma lesions in this study may have been due to delayed effects of antiretroviral therapy on Kaposi's sarcoma growth. Although no new halofuginone studies are being planned by the AMC, information gained from this study provides important clues that will influence future studies of topical non-absorbed agents for the treatment of Kaposi's sarcoma.

Reference: Koon HB, Fingleton B, PhD, Lee JY, et al. Phase II AIDS Malignancy Consortium Trial of Topical Halofuginone in AIDS-Related Kaposi Sarcoma. *J Acquir Immune Defic Syndr* 2011;56:64-68. http://www.ncbi.nlm.nih.gov/pubmed/21068672

AIDS Malignancy Consortium Trial # 036: Phase II Trial of Topical Halofuginone in Patients with HIV-related Kaposi's Sarcoma

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For more information about HIV cancer malignancies, visit the AMC Web site at

http://www.AIDSCancer.org