



UCLA Research Study AMC 036

A Phase II Trial of Halofuginone in Patients With HIV Related Kaposi's Sarcoma

Study Purpose and Design

The purpose of this study is to evaluate the effects of halofuginone 0.01% ointment, when applied directly to Kaposi's sarcoma skin lesions. Halofuginone is an investigational drug that has had anticancer effects in animals. It interferes with certain proteins, including collagen and matrix metalloproteinases that are involved in the growth of cancer cells, including Kaposi's sarcoma.

Twelve KS lesions on the skin will be picked out by the study doctor and will be divided into two groups of 6 lesions each. One group will be called Group A and the other will be called Group B. You will be given two tubes of ointment marked as "A" and "B". One tube will have an ointment containing halofuginone at a concentration of 0.01%. The other tube will have the same ointment without halofuginone. Neither the patient nor the treating doctors and nurses will know which tube has the ointment containing halofuginone and which tube has the ointment without halofuginone. Patients will be instructed to put the ointment in tube "A" on the lesions in Group A and the ointment in tube "B" on the lesions in Group B twice a day for 12 weeks. At the end of 12 weeks, if the treated lesions in either group A or group B are stable or improved, and if no dangerous side effects have been seen, there will be the opportunity to apply halofuginone 0.01% ointment twice a day to all of the remaining Kaposi's lesions in Group A and Group B for another 12 weeks. Biopsies of two skin lesions will be performed before starting treatment, after 4 weeks of treatment and after 12 weeks of treatment. Kaposi's sarcoma lesions will be measured and counted every 4 weeks, and routine and research blood tests will be done every 4 weeks while on study. **The maximum time on study will be 24 weeks.**

Inclusion Criteria

- Biopsy proven Kaposi's sarcoma (KS).
- At least 14 KS lesions on the skin
- HIV-positive and 18 years of age or older.
- Hemoglobin at least 8g/dl, absolute neutrophil count at least 750 cells/mm³ and platelet count at least 75,000/mm³.
- Liver enzymes no more than 3 times normal.
- If on antiretroviral treatment, the treatment regimen must be stable for at least 12 weeks.

Exclusion Criteria

- Kaposi's sarcoma in internal (visceral) organs.
- Severe swelling (edema) from KS.
- Need for chemotherapy for KS or other cancers, or any chemotherapy within 4 weeks of starting treatment with halofuginone.
- Pregnant or nursing women cannot participate.
- Active untreated opportunistic infection.

Principal Investigator

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For more information, call 310.206.6414