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MEETING ABSTRACTS Open Access

Phase IIA trial of 1% topical cidofovir for treatment of high-grade perianal squamous intraepithelial neoplasia in HIV-infected men and women (AMC046)

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Objective

Treatments for high-grade perianal intraepithelial neoplasia (PAIN 2-3), include surgical ablation/excision and have significant morbidity and recurrence rates. Cidofovir, a cytidine nucleotide analogue, has broad-spectrum antiviral activity. This multicenter study prospectively evaluated the efficacy, safety, and tolerability of topical cidofovir for treatment of PAIN 2-3 in HIV-positive individuals.

Methods

HIV-positive patients with biopsy-proven PAIN 2-3 ≥ 3 cm^2 were eligible. Subjects applied 1% topical cidofovir for 6 two-week cycles consisting of 5 consecutive days of treatment and 9 days without treatment. Subjects were evaluated every 2 weeks. High-resolution anoscopy and biopsy were performed 6 weeks after the last cycle. Results were scored as stable disease (SD), partial response (PR) (> 50% reduction in size), complete response (CR), or progressive disease (PD) based on size and histology.

Results

24 men and 9 women were enrolled. Mean age was 33 years, median HIV RNA level was <75 copies/ml, and mean CD4 count was 440/μl. HPV DNA was detected in intra-anal swabs of 31 of 32 (97%) subjects with analyzable specimens. The most common type was HPV16 (44%).

27 (82%) subjects completed treatment per protocol—CR: 4 (15%); PR: 12 (44%); SD: 9 (33%); PD: 2 (7%) (1 with a superficially invasive cancer and 1 with new PAIN 2-3). Six subjects did not complete treatment because of discomfort (1), poor compliance (4), and CR after 4 cycles (1).

26 of 33 subjects (79%) reported adverse events likely related to treatment. Most were mild or moderate, including self-limited, localized, superficial ulcerations in the disease area (2 mild, 19 moderate, 1 severe), discomfort (4 mild, 14 moderate), itching (1 mild, 3 moderate), and bleeding (6 mild). Seven (21%) had mild transient proteinuria.

Conclusions

Topical cidofovir is a well-tolerated and effective treatment for PAIN 2-3 in HIV-positive patients. A larger study is warranted.

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