

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)

Elizabeth A Stier<sup>1\*</sup>, Stephen E Goldstone<sup>2</sup>, Mark H Einstein<sup>3</sup>, Naomi Jay<sup>4</sup>, J Michael Berry<sup>4</sup>, Timothy Wilkin<sup>5</sup>, Jeannette Lee<sup>6</sup>, Lori Panther<sup>7</sup>, David Aboulafia<sup>8</sup>, Joel Palefsky<sup>4</sup>

From 12<sup>th</sup> International Conference on Malignancies in AIDS and Other Acquired Immunodeficiencies (ICMAOI)  
Bethesda, MD, USA. 26-27 April, 2010

## 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  $\geq 3$  cm<sup>2</sup> 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/ $\mu$ l. 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.

## Acknowledgements

This article has been published as part of *Infectious Agents and Cancer* Volume 5 Supplement 1, 2010: Proceedings of the 12<sup>th</sup> International Conference on Malignancies in AIDS and Other Acquired Immunodeficiencies (ICMAOI). The full contents of the supplement are available online at <http://www.biomedcentral.com/1750-9378/5?issue=S1>.

## Author details

<sup>1</sup>Department of Obstetrics and Gynecology, Boston University Medical Center, Boston, MA, USA. <sup>2</sup>Department of Surgery, Mount Sinai School of Medicine, New York, NY, USA. <sup>3</sup>Montefiore Medical Center and Einstein Cancer Center, Bronx, NY, USA. <sup>4</sup>Department of Medicine, University of

\*Correspondence: [elizabeth.stier@bmc.org](mailto:elizabeth.stier@bmc.org)

<sup>1</sup>Department of Obstetrics and Gynecology, Boston University Medical Center, Boston, MA, USA

Full list of author information is available at the end of the article

California, San Francisco, San Francisco, CA, USA. <sup>5</sup>Division of Infectious Diseases, Weill Cornell Medical College, New York, NY, USA. <sup>6</sup>Department of Biostatistics, University of Arkansas for Medical Sciences, Little Rock, AR, USA. <sup>7</sup>Division of Infectious Diseases, Beth Israel Deaconess Medical Center, Boston, MA, USA. <sup>8</sup>Division of Hematology and Oncology, Virginia Mason Medical Center and Department of Hematology, University of Washington, Seattle, WA, USA.

Published: 11 October 2010

doi:10.1186/1750-9378-5-S1-A60

**Cite this article as:** Stier *et al.*: Phase IIA trial of 1% topical cidofovir for treatment of high-grade perianal squamous intraepithelial neoplasia in HIV-infected men and women (AMC046). *Infectious Agents and Cancer* 2010 **5**(Suppl 1):A60.

**Submit your next manuscript to BioMed Central  
and take full advantage of:**

- Convenient online submission
- Thorough peer review
- No space constraints or color figure charges
- Immediate publication on acceptance
- Inclusion in PubMed, CAS, Scopus and Google Scholar
- Research which is freely available for redistribution

Submit your manuscript at  
[www.biomedcentral.com/submit](http://www.biomedcentral.com/submit)

