Pomalyst Clears Anal Cancer Precursor in Small Study

Immune modulator demonstrated an overall response rate of 52%

March 14, 2020 By Liz Highleyman

Pomalyst (pomalidomide), currently approved for multiple myeloma, led to the clearance of precancerous anal lesions caused by human papillomavirus (HPV) in HIV-positive and HIV-negative men, according to a recent study.

Just over half of study participants experienced complete or partial resolution of high-grade anal lesions after they were treated with Pomalyst for six months, rising to 63% after an additional six months of post-treatment follow-up.

Mark Polizzotto, MD, of the Kirby Institute at the University of New South Wales in Sydney, presented the findings from the Phase II SPACE Study, which evaluated low-dose Pomalyst as a treatment for high-grade anal lesions.

The results were presented virtually at the Conference on Retroviruses and Opportunistic Infections last week, after the in-person meeting in Boston was cancelled due to the coronavirus crisis.

Anal cancer and its precursor, anal dysplasia (abnormal cell and tissue growth), are more common among people living with HIV compared with the general population. Among gay and bisexual men, the rate is around one in 1,000—comparable to that of colon cancer in the general population, Polizzotto noted as background.

Anal dysplasia and cancer are usually caused by high-risk HPV types including 16 and 18, which are common in both HIV-positive and HIV-negative gay and bi men. HPV also causes cervical, oral and other cancers.

People typically first develop less severe dysplasia, known as low-grade squamous intraepithelial lesions (LSIL), which can progress to more severe high-grade squamous intraepithelial lesions (HSIL) and eventually invasive carcinoma. However, dysplasia may resolve on its own, LSIL does not always lead to HSIL and HSIL does not always lead to cancer, so the best course of care is unclear. HSIL is typically treated with local therapies but recurrence is common.

Pomalyst interferes with angiogenesis (blood vessel growth) and appears to enhance immune response against the HPV E6 protein. A systemic T-cell response is associated with spontaneous HSIL clearance of HSIL, Polizzotto noted. The drug has also shown activity against Kaposi sarcoma, a cancer caused by a herpesvirus.

The study included 10 HIV-positive and 16 HIV-negative men; the latter group was somewhat younger (median 48 versus 58 years, respectively). Almost all were white. All of the men living with HIV were on antiretroviral therapy with full viral suppression and the median CD4 T-cell count was high.

All participants had Grade 3 HSIL, which had lasted at least one year and a median of three years. Just over half had HPV type 16 and half had multiple high-risk HPV types. Smoking—a known risk factor for anal cancer—was common, reported by 40% in the HIV-positive and 31% in the HIV-negative group.

Participants received low-dose oral Pomalyst for 21 days in each 28-day cycle for a total of six cycles. They were then were followed off treatment for an additional six months. The men also received aspirin or another medication to reduce the risk of blood clots, a possible side effect of the drug.

At the end of treatment, among the 24 evaluable participants, the combined overall response rate was 52%, including eight (35%) with complete remission and four (17%) with partial responses, defined as at least a 50% reduction in lesion size. The overall response rates were 56% in the HIV-positive group and 50% in the HIV-negative group.

At the end of the extended follow-up period, the combined overall response rate was 63%, including eight (33%) complete responders and seven (29%) partial responders. The extended overall response rates were 67% in the HIV-positive group and 60% in the HIV-negative group.

Analysis of immune responses showed activation of various subsets of CD4 (helper) and CD8 (killer) T cells, as well as CD4 responses against the HPV E6 protein.

Treatment was generally safe and well tolerated. The most common adverse events were neutropenia (low white blood cell count), constipation, fatigue and skin rash, most of which were mild. Side effects were comparable in the HIV-positive and HIV-negative groups. There were three serious adverse events, including a cardiac event deemed to be possibly drug-related. No one stopped treatment due to side effects.

“Low dose pomalidomide was feasible and well tolerated in individuals with HSIL,” the researchers concluded. “Further study in HPV-associated premalignancy is warranted.”

Click here to read the study abstract.

Click here to learn more about anal cancer.

© 2026 Smart + Strong All Rights Reserved.
http://beta.docker.cancerhealth.com/article/pomalyst-clears-anal-cancer-precursor-small-study