Topical Cantharidin Revisited: A Phase 2 Study Investigating a Commercially-Viable Formulation of Cantharidin (VP-102) for the Treatment of Molluscum Contagiosum

Anthony K Guzman MD, Jessica L Garelik DO, Steven R Cohen, MD, MPH

1Department of Internal Medicine, Division of Dermatology, Albert Einstein College of Medicine, Bronx, NY.

Background: Molluscum contagiosum (MC) is a common cutaneous infection caused by a DNA poxvirus, predominantly affecting children. There is a paucity of high quality evidence on which to make clinical decisions in treating MC. (1) Cantharidin, a topical vesicant historically derived from a blister beetle, is a commonly-used treatment for this condition. However, despite the prevalence of its use, cantharidin is not FDA-approved, is not standardized in formulation or treatment regimen and is not always manufactured in accordance with Good Manufacturing Practices (GMP), leading to a lack of commercial availability. (2,3)

Objective: To determine the efficacy and safety of VP-102, a novel, standardized, commercially-viable cantharidin formulation produced under GMP for the treatment of MC.

Methods: We conducted a 12-week, open-label pilot trial at a single outpatient dermatology clinic. Subjects 2-17 years (n = 30) with a clinical diagnosis of MC and < 50 lesions were included. Subjects were treated with a single-use vial containing a standardized 0.7% w/v cantharidin solution, produced under GMP (VP-102), applied with the wooden end of a cotton swab approximately every 21 days, for up to 4 treatments or until complete lesion clearance. Subjects were instructed to wash treatment off all lesions at either 6 hours (Cohort 1: 14/30, 46.7%) or 24 hours (Cohort 2: 16/30, 53.3%), or earlier if significant blistering occurred. Lesion counts and adverse events (AEs), including local skin reactions, were documented at each visit. Quality of life was also measured using the Children’s Dermatology Quality of Life Index (CDLQI) at baseline and at the end of study (EOS). The primary endpoint was the percentage of subjects achieving total clearance by EOS on Day 84.

Results: The mean patient age was 5.8 (range= 2-12 yrs). A total of 26 subjects (86.7%) experienced at least one expected local skin reaction such as blistering or erythema. No serious or unexpected treatment-related AEs were encountered. A total of 25 subjects pooled from both cohorts completed the study. Eleven subjects (44.0%) achieved total lesion clearance by EOS. The mean ± SD lesion count was significantly reduced from 23.0 ± 15.6 at baseline to 6.8 ±
11.7 at EOS (p < 0.0001). The mean CDLQI score was markedly improved from 3.9 ± 5.6 at baseline to 0.38 ± 1.3 at EOS (p = 0.01).

**Conclusions:** VP-102 was well-tolerated with either a 6 or 24-hour exposure and was associated with a significantly reduced lesion count, improved quality of life and complete clearance of MC lesions in nearly half of the patients.

**Figure 1:** Lesion clearance after VP-102.

![Lesion Clearance After VP-102](image)

**References:**