

A Phase II Single Arm Open-Label Clinical Trial of Chemotherapy of BCCs with Tazarotene 0.1% in Subjects with Basal Cell Nevus Syndrome

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Synopsis:

The purpose of this multicenter, open-label trial is to evaluate the effect of topical tazarotene cream on basal cell carcinomas (BCCs) in subjects with basal cell nevus syndrome (BCNS). Men and women 18-75 years of age who have at least one clinically diagnosed BCC greater than or equal to 3 mm in diameter (target lesion) on any area of the skin except the face, chest, and back (and not impinging on vital sites) and meet the diagnostic criteria for BCNS will be eligible. Participants (n=26 - 42) will be treated with 0.1% tazarotene qd topically applied to the face and the target lesion for 18 months, with observational follow-up (no treatment) for an additional 18 months. History and physical, skin examinations and photography, adverse event review and drug dispensing and compliance will be performed at three month intervals; fasting blood samples will be collected at months 0, 12, 24, and 36 for research analysis and whole blood and plasma banking; pregnancy testing and counseling will be performed at each clinic visit through month 18 in women of childbearing potential; local clinic examinations will also continue during the study for clinical care as needed. In addition, adverse events will be monitored throughout the study by telephone contact at six week intervals. There is no regular scheduled follow-up visit for subjects after cessation of treatment but all adverse events will be followed until resolution. The primary efficacy endpoint for this trial is the proportion of complete responses (defined as disappearance of the targeted BCC during the 18 months of treatment and failure to recur during the ensuing 18 months without treatment); secondary efficacy endpoints include time to lesion clearance, time to progression, estimated duration of complete response, and overall response of treated lesions, and the tertiary chemoprevention endpoint is the incidence of new BCCs on the face.

The goal of this study is to expand and refine chemotherapeutic strategies in individuals with basal cell nevus syndrome (BCNS) who are at high risk for the development of basal cell carcinomas (BCCs). Our hypothesis, based on preclinical mouse data and on small human chemotherapy trials, is that application of tazarotene to susceptible skin regions will reduce the development of new basal cell carcinomas. The specific objective of the study is to determine whether Tazarotene at a dose of 0.1% administered topically over a period of 18 months as a chemotherapeutic agent in subjects with BCNS, will successfully treat the targeted BCC. During the treatment period (month 0-18), the safety and efficacy of 0.1% Tazarotene cream will be compared with that of the untreated months 18-36. Daily application of the study medication for 18 months will be for the treatment and prevention of basal cell carcinomas. Evaluations of the face and target lesion will be done at the Study Centers at 3 month intervals from months 0-36. The primary efficacy end point is the proportion of complete response (complete disappearance of the targeted BCC after treatment) among all treated patients.