Diphencyprone Immunotherapy in Cutaneous Neurofibromas

Neurofibromatosis type 1 (NF1) is the most common genetic tumor predisposition syndrome which is characterized by multiple benign tumors, including cutaneous neurofibromas (CNs). These tumors are benign but can pose significant challenges to patients due to their size, pain, and the need for frequent treatments. One treatment option for CNs is surgery or laser therapy; however, only a limited number of tumors can be surgically removed. Topical treatments for CNs include cryotherapy, laser therapy, and xerotherapy, but these treatments can be painful and may not completely eliminate the tumors. Diphencyprone (DPCP) has been used to eliminate tumors of the human skin, but its use is limited due to the high risk of skin reactions.

In this study, we are planning a phase 1 clinical trial for DPCP in patients with CNs. The goals of the trial are to evaluate the safety and efficacy of DPCP in treating CNs, and to determine the optimal dose and treatment regimen. The study will involve patients with CNs who have not responded to other treatments or who are not suitable for surgery or laser therapy.

The study will be conducted in a randomized, double-blind, placebo-controlled trial. Patients will be randomly assigned to one of three treatment groups: DPCP alone, placebo alone, or DPCP plus placebo. The primary outcome measure will be the reduction in tumor size and the number of new tumors over a 12-month period.

The study will be conducted at multiple centers across the United States and enroll up to 40 patients. The study will be monitored by an independent data safety monitoring board to ensure patient safety.

The results of this study will provide important information about the safety and efficacy of DPCP in treating CNs and may lead to the development of new treatment options for this common condition.