Clinical Trial Eligibility Checklist:

- ☐ 14 to 30 years of age
- ☐ Diagnosis of Neurofibromatosis Type 1 by current diagnostic criteria
- ☐ Tumor Location: cutaneous, trunk, or limbs only
- ☐ Tumor Type: superficial dermal neurofibromas

- ☐ Absence of any other malignancy or chemotherapy within 6 weeks prior to planned study treatment
- ☐ Does not have porphyria (a skin condition)
- ☐ Life expectancy more than 3 years
- ☐ Not pregnant and willing to abstain or take precautions to avoid pregnancy during trial
- ☐ Not allergic to sunlight, aminolevulinic acid HCL, rubbing alcohol, laureth-4 polyethylene glycol or ultrasound gel.
- ☐ Not currently undergoing any other neurofibromatosis treatment
- ☐ Willing and able to comply with study follow-up requirements

Doctors Conducting this Trial

* Harry Whelan MD, Neurologist
* Donald Basel MD, Geneticist
* Tejaswini Deshmukh MD, Radiologist
* Edit Olasz MD, Dermatologist
* Dawn Siegel MD, Dermatologist
* Nghia Vo, MD, Radiologist

Why is this study being done?

The purpose of this clinical trial is to learn more about the effects of topical application of Levulan Kerastick on the growth rate of Type 1 neurofibromas (NFs).

Summary

Each enrolled participant will have 1 application of an experimental medication combined with a red light shown on some of their neurofibromas. Then every 6 months for 3 years, they will have examination and measurements taken of the NFs being studied.

Additional information can be found at : https://clinicaltrials.gov/ct2/show/NCT02728388?

Scan code below for more study information

Scan me

V.9.23.19

Neurofibromatosis Type 1

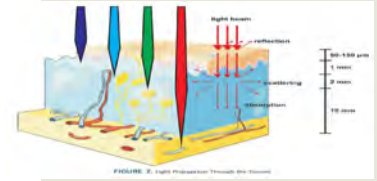
Photodynamic Therapy Phase II Study Utilizing Levulan® Kerastick®

What is Levulan® Kerastick®?

Levulan®, also called aminolevulinic acid hydrochloride (HCL), is a chemical that has been approved by the Food and Drug Administration (FDA) as an effective treatment in adults with a skin condition known as actinic keratosis. Kerastick® is the name given to the pen-like container in which the Levulan is stored. The pen-like design allows for easy, precise drug administration by the research doctor.

In this research , the study doctor will choose several neurofibromas (NFs) of similar size. Each participant will have Levulan® applied to some NFs. Some other NFs will have only the solution that the drug is normally mixed applied (a placebo).
The next day, all the NFs being studied will have an FDA -approved red light shown on them.

The drawing of a section of skin, (below) , shows that red light goes deeper into the skin than other types of light.



Phase I study results showed that Levulan® was absorbed by the NFs to which it was applied. It was not absorbed by the surrounding skin. Some NF cells that absorbed Levulan® died. These results were shared in a poster presentation at the Children's Tumor Foundation International Neurofibromatosis Meeting by the investigators listed above. (*Riccardi et al, 9/2019*).

Why is this Phase 2 study being done?

Neurofibromatosis not only cause physical changes in the skin, they also can be painful , and negatively affect physical functioning and emotional health.

There is currently no FDA-approved treatment for slowing the growth of neurofibromas. (NFs). While the Phase 1 study showed some cell death occurring in the treated NFs, the FDA requires this treatment to be studied in additional controlled trials before being considered for FDA approval. **This study will collect important information about side effects of the combination experimental Levulan® plus light therapy and how to manage side effects.**

What is the long-term goal of this research?

This research program is providing new information about how safe and effective the application or Levulan® Kerastick® combined with red light are at slowing the growth of Type 1 Neurofibromas.

This trial is sponsored by:

Ben's Research Fund

http://bensresearchfund.org

Bleser Family Foundation

MEDICAL COLLEGE OF WISCONSIN

Children's Hospital of Wisconsin Research Institute

Study Details:

- **Study participation is voluntary.** Participants are asked to commit to being in the study for 3 years. However, you or the study doctor have the right to stop your participation at any time.
- There is no guarantee of personal benefit from study participation.
- Before you participate, the researchers will contact your regular healthcare team to ensure that you meet the study inclusion criteria and that being in the study will not interfere with your regular care.
- While in the study, you will continue your regular care with your current health care providers.
- At the first 2-day visit, study drug and light therapy treatments are given. That is the only time study drug is given. Participants return about every 6 months (5 more times) for 3-hour Follow-up Visits over 3 years to monitor NF growth.
- All costs of study will be covered by the study. You or your insurance will not be responsible for any study procedures.
- Travel reimbursement is available for participants. The amount and type of transportation/travel funding depends on how far participants live from the Medical College of Wisconsin Milwaukee Cam-

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