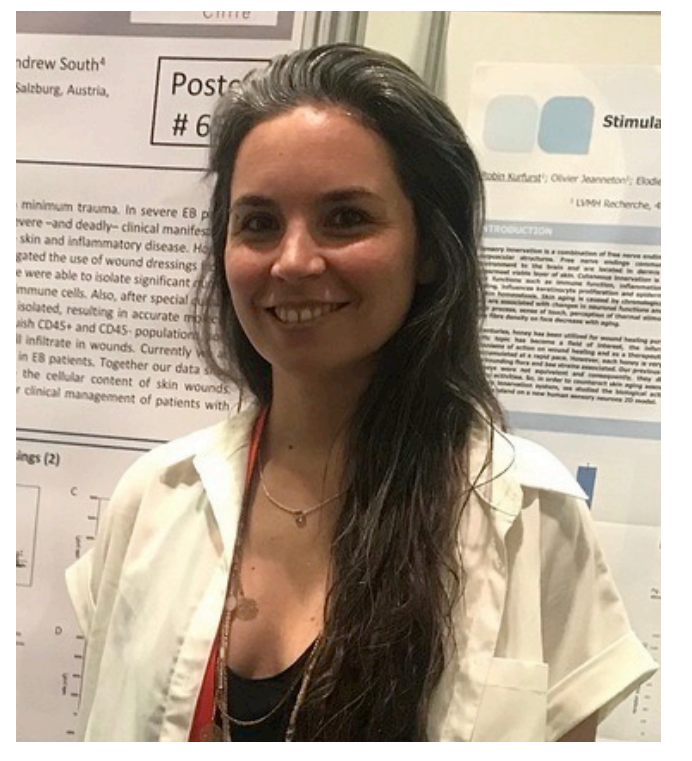


Repurposing a Common Antioxidant Nutraceutical, N-Acetylcysteine, for Wound Treatment in Butterfly Skin



Ignacia Fuentes PhD
DEBRA Chile
EB and Molecular Biology



Francis Palisson MD
DEBRA Chile
Internal Medicine and Dermatologist



Belkis Noya MD
DEBRA Chile
Dermatologist



María Joao Yubero MD
DEBRA Chile
Pediatrician and Infectologist



Andrew South PhD
Thomas Jefferson University
Fibrosis and Skin Cancer in EB

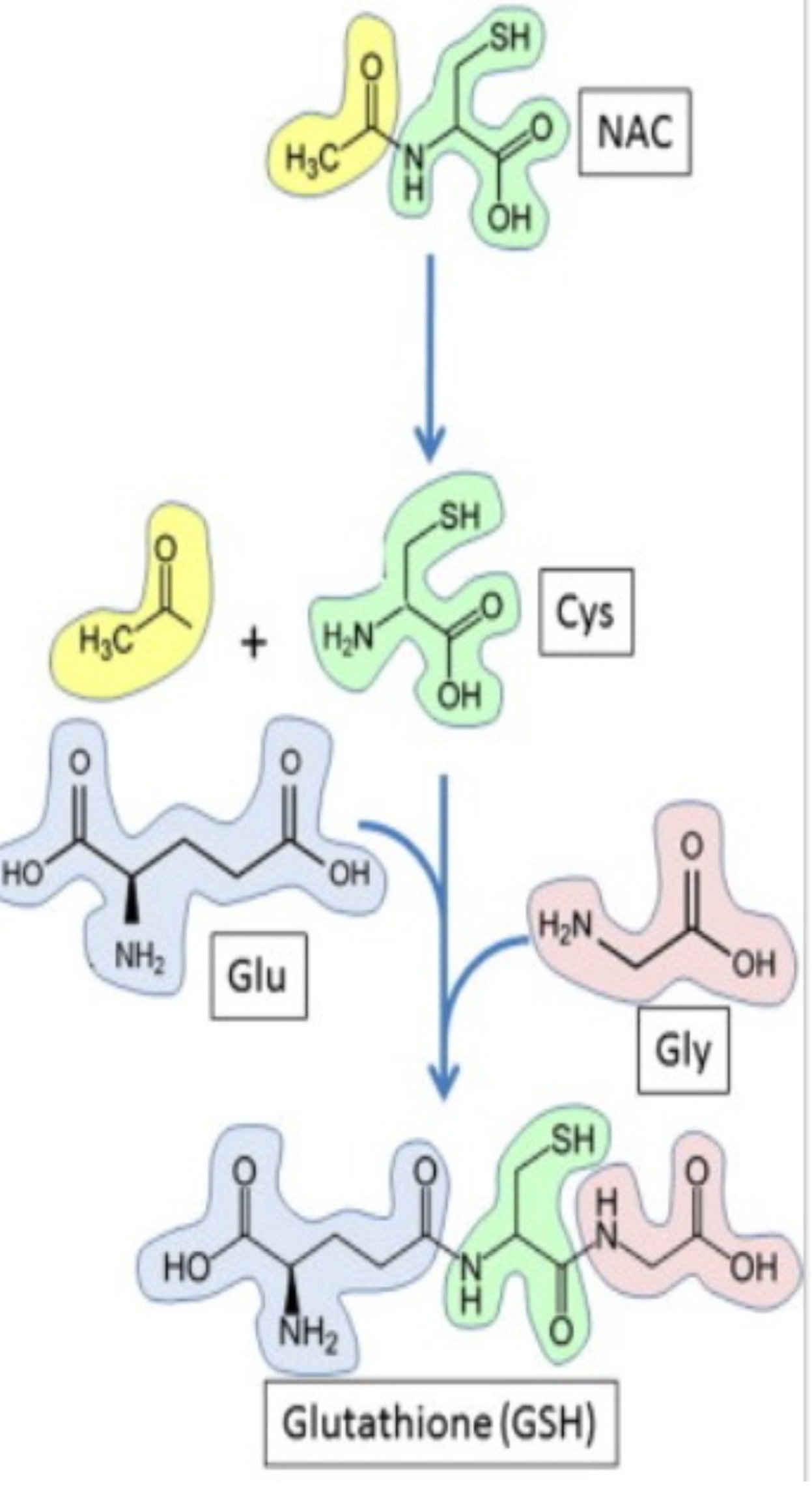
PROPOSED TREATMENT

Repurposing topical N-Acetylcysteine (NAC) to accelerate and dramatically improve healing of chronic wounds in RDEB patients.

NAC is a molecule that has a long history of treating acetaminophen overdose. Glutathione (GSH), is one of the most well-studied antioxidants. When free NAC enters a cell, it can lead to GSH production, increasing cellular protection against oxidative stress. NAC is currently involved in multiple research studies proving its antioxidant effects and its ability to improve inflammation, fibrosis, and endothelial dysfunction.

Previously, the effects of wound healing of NAC has been demonstrated in mice and rats. Other studies have shown the clinical benefits of topical NAC in skin diseases including acne, lamellar ichthiosis, and pressure ulcers.

This project is a proof-of-concept trial investigating if topical NAC can be beneficial for chronic wound healing in RDEB patients. The information obtained from the study also could help assess the usefulness of NAC in broader wound healing applications.



SUMMARY STATEMENT

An 8-patient trial investigating topical N-Acetylcysteine (NAC) to improve healing of chronic wounds in the skin-fragile genetic condition recessive dystrophic Epidermolysis bullosa (RDEB)

DISEASE/CONDITION

Epidermolysis Bullosa (EB), also known as “Butterfly Skin,” is a rare inherited disease that makes skin so fragile that it can tear or blister at the slightest touch.

One of the most severe EB forms is recessive dystrophic EB (RDEB). Patients suffering from this disease have excessive skin and mucosal fragility that produces painful blisters with minor trauma and often results in chronic or hard to heal wounds. They also suffer from chronic pain and itch. Severe forms of RDEB can develop even more serious complications, including:

- Severe anemia
- Malnutrition
- Fibrosis
- Recurrent infections
- Aggressive skin cancer



CURRENT TREATMENT

Currently there is no specific treatment for RDEB, only Palliative Care. Wounds require debridement, topical antiseptics and proper dressings that promote healing and/or reduce pain.

An early treatment for skin inflammation and fibrosis in RDEB wounds is necessary to improve healing and decrease the multiple complications associated to non-healing wounds.

There is a need for a simple, easy-to-implement, safe and cheap treatment for EB patients.

PROJECT

A proof-of-concept clinical trial to explore the efficacy and tolerance of topical NAC in combination with a non-adhesive wound dressing in RDEB patients.

This single site, open-label, prospective, controlled clinical trial will recruit 8 RDEB patients age 2-18 years old. We will select 2 independent wounds per patient, for a total of 16 wounds. One-half of each wound will received the topical NAC, one-half will treated with placebo, and the entire wound will be covered in non-adhesive dressing.

Topical NAC will be applied in the same half wound at each wound dressing change, including center and home visits. Patients will be monitored and evaluated weekly for 28 days.

Specific Aims

- To verify the safety and feasibility of this treatment
- To observe whether topical NAC improves RDEB wound healing, by either reducing the time to heal or increasing re-epithelialization speed



We also expect that topical NAC may improve other common complications of chronic wounds, such as pain and itch. For patients, a reduction on these complications and faster wound healing will certainly result in a positive and significant impact on their quality of life.



CUREACCELERATOR®
Live!

for Rare Diseases June 10, 2021

