

Subject: FW: February 2020 Research Update

Hi First name,

I am delighted to share some research news with you in January. The year has just kicked off and already scientific development is hitting the headlines.



EB World Congress 2020 (EBWC2020)

Jimmy Fearon (CEO), Cheryl Gunning and I attended the EB World Congress in London last month. 600 people attended the event. It was a chance to share global research and management.

Topics included research from understanding the biology of the condition, to patient engagement.

EB Therapies in the Headlines in 2020

The 2020 EB World Congress highlighted the volume of therapies being developed to fight EB. These are just some that hit the headlines since the beginning of the year.

Abeona Launches Phase 3 Trial Testing EB-101 Cell Therapy for RDEB

Abeona Therapeutics has begun its **Phase 3** VIITAL trial evaluating EB **corrected, skin graft**, cell therapy for the treatment of **RDEB**. The trial at Stanford Medicine and additional sites are expected to open over the next few months. For more information [here](#).



InMed to Advance Cannabinol (CBN) into Therapeutic Clinical Trials

InMed Pharmaceuticals has received regulatory approval to start a **Phase 1** trial in the Netherlands to evaluate the treatment candidate INM-755 for **EB**. INM-755 is a **topical** therapy designed to address the symptoms associated with EB and other skin conditions. It is designed to work by boosting the levels of keratin, a key structural protein in the outer layers of the skin, hair, and nails. Preclinical studies, presented at EBWC2020, suggested that the experimental therapy may ease inflammation, itching, and pain in EB patients.



InMed to Advance Cannabinol (CBN) into Therapeutic Clinical Trials

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Aegle Raises \$4M to Fund First Phase 1/2 Trial of Experimental Therapy for DEB



Aegle Therapeutics has raised \$4 million to fund the first clinic people with **DEB**. It is based on the company's extracellular **transporters and delivers type VII collagen** protein as well as fibroblasts stimulating the cells to produce their own type VII collagen.

The **Phase 1/2** trial is anticipated to begin in the first half of 2020. The study will be conducted in the U.S. and is expected to include 30 participants. More information [here](#).



Spray Therapy APR-TD011 Earns FDA Orphan Drug Designation to Treat EB

(FDA) has granted orphan drug status (reduced development costs) to APR-TD011, a therapy for **EB**.

The therapy is administered as an easy-to-use spray formulation allowing the treatment of skin wounds, while avoiding skin contact and contamination.

Psoriasis Ointment Helped with Itch, Healing in Phase 2 EB Study



A small, placebo-controlled, **Phase 2** study using low-dose calcipotriol showed a significant and steady decrease in itch in people with **RDEB**. Calcipotriol is a Vitamin D analog. Vitamin D is a critical factor in wound healing. Calcipotriol is a drug to treat psoriasis, at a very high concentration. Dr Christina Guttman-Gruber led the research at EB House, Austria. The next step will probably be to plan a multicenter trial of this treatment, subject to funding.

Global Fight Against EB - EB Registry -

The development of the **Irish and international EB registries** was presented at the EBWC2020 by **Godfrey F International Skin Registry Solutions (NISR)**. A registry relevant data about the health and medical treatment of people collecting and analysing information on *all* people with EB in order to understand their health and wellbeing, and the treatments that



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Godfrey explained the importance of registry data in the global understanding of a disease and in the development of new therapies.

Get Involved in Research

We recently participated in a forum with other charities and industry, organised by Health Research Charities Ireland. This forum emphasised the importance of the patient voice in the development of therapies and ensuring that industry is using the patient experience, living with a condition, in their developments. In a lot of cases, industry does not talk to patients until clinical trials, by which time the therapy it has developed may not fully meet patients needs. We are trying to encourage industry to involve patients throughout therapy developments to help guide on patients needs.

If you are interested in hearing more on this and getting involved through our Patient Involvement Panel please contact me.

Your help is vital in developing treatments for all forms of EB. You can get involved in many ways including:

Patient Involvement Panel (PIP)

- Participate in research studies
- Advise on DEBRA research
- Participate in research communication

Facebook - [DEBRA International Research Involvement Network](#)

- Targeted at people with EB who want to learn more and get involved in EB research.
- Participate in assessments and questionnaires.
- Get up to date information on current research.

Contact me - Sinead.hickey@debraireland.org

- What would you like to see in our research newsletter?
- What would you like to see on our website?