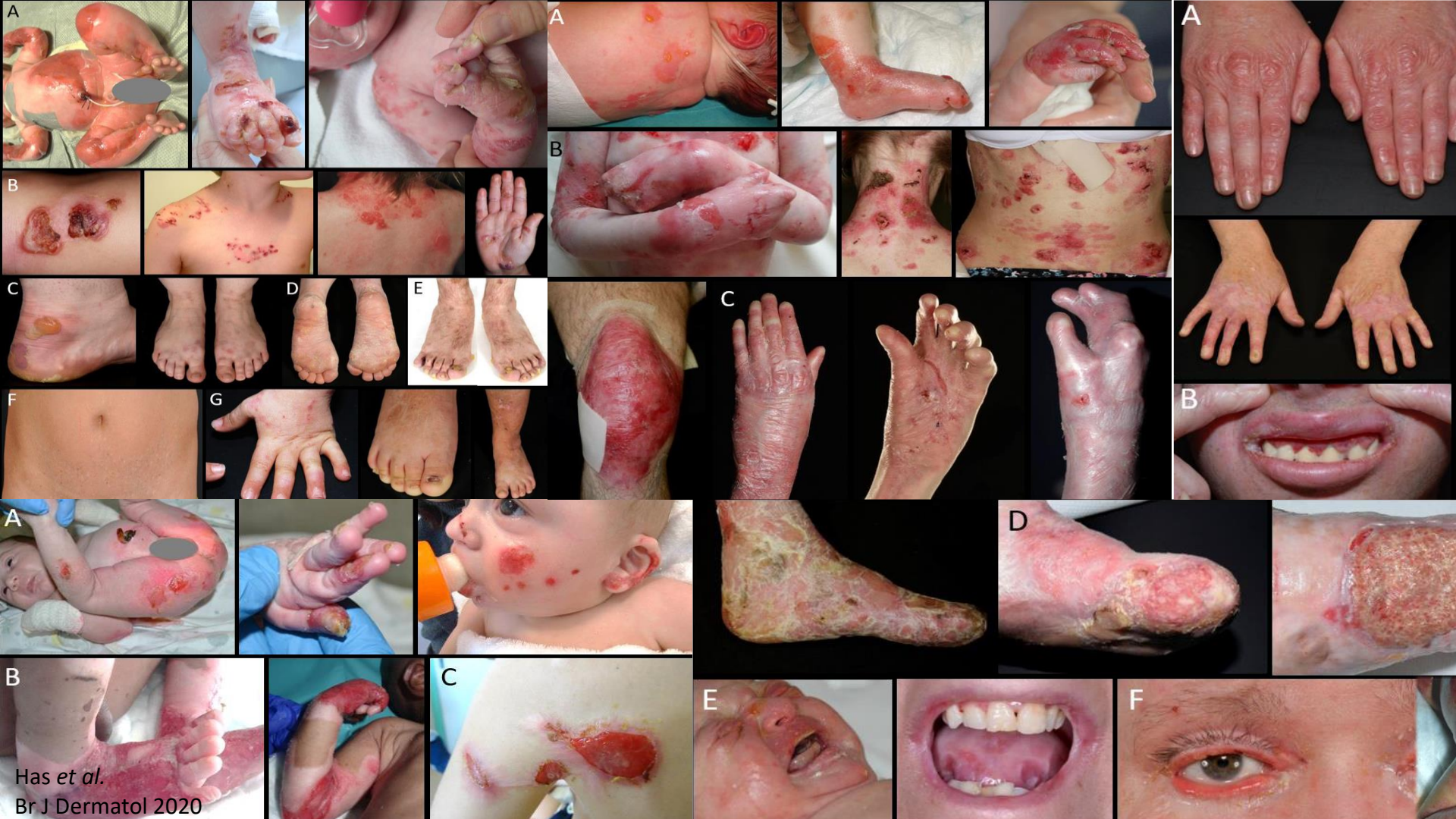
Clinical Trials in EB

John McGrath

(on behalf of Jakub Tolar)



Has *et al.*
Br J Dermatol 2020

Clinical trials.gov October 2024

131 studies on EB listed

24 studies recruiting

Allogeneic ABCB5-positive Dermal Mesenchymal Stromal Cells for Treatment of Epidermolysis Bullosa (Phase III)

ClinicalTrials.gov ID
NCT05838092

The aim of this clinical trial is to investigate the safety and efficacy of allo-APZ2-OTS administered intravenously to subjects with recessive dystrophic epidermolysis bullosa (RDEB) compared to placebo

RECRUITING

Allogeneic ABCB5-positive Dermal Mesenchymal Stromal Cells for Treatment of Epidermolysis Bullosa (Phase III, Cross-over)

ClinicalTrials.gov ID
NCT05464381

The aim of this clinical trial is to investigate the safety and efficacy of allo-APZ2-OTS administered intravenously to subjects with recessive dystrophic epidermolysis bullosa (RDEB) compared to placebo. An additional baseline-controlled open-label arm will be included to investigate the safety and efficacy of allo-APZ2-OTS administered intravenously to subjects with JEB and to RDEB subjects < 1 year

RECRUITING

Characterization of the Microbiome in Colonized Dystrophic and Junctional Epidermolysis Bullosa Wounds Before and After Use of APR-TD011 ® Spray Solution

ClinicalTrials.gov ID
NCT05533866

Hypochlorous acid

Characterization of the Microbiome in Colonized Dystrophic and Junctional Epidermolysis Bullosa Wounds Before and After Use of APR-TD011 ® Spray Solution

ENROLLING BY INVITATION

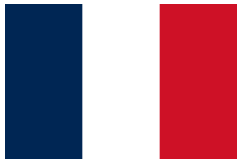
CACIPLIQ20 in Wound Healing in Subjects With Epidermolysis Bullosa (MATHBULL)

Heparin Sulphate PG matrix

ClinicalTrials.gov ID
NCT06007235

The goal of the MATHBULL study is to confirm preliminary observations (PAIN AND WOUND HEALING) in a placebo-controlled double-blind pilot study. The results of this pilot study will help to design a pivotal study.

RECRUITING



A Natural History Study of Corneal Abrasions in Patients With Dystrophic Epidermolysis Bullosa (DEB)

ClinicalTrials.gov ID
NCT06563414

This study is a non-interventional, observational study that will evaluate the natural history of corneal abrasions in patients with Dystrophic Epidermolysis Bullosa (DEB). Corneal abrasion symptomology, frequency, and outcomes will be evaluated for up to 6 months

RECRUITING

EB-101 Treatment for New and Previously Treated Patients With Recessive Dystrophic Epidermolysis Bullosa (RDEB)

ClinicalTrials.gov ID
NCT05725018

To evaluate and further characterize the safety of EB-101 (LZRSE-Col7A1 gene-corrected keratinocyte sheets with type VII collagen [C7] expression) for the treatment of large, chronic RDEB wounds in new and previously EB-101 treated patients 12 months and older.

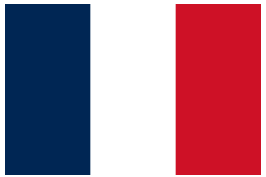
RECRUITING

Impact of Complex Care Training of Hereditary Epidermolysis Bullosa on Caregiver Burden (FIREB)

ClinicalTrials.gov ID
NCT05248503

The burden on parents is heavy, assessed by specific scales, but to date there are no studies examining the impact of epidermolysis bullosa care on caregiver stress

RECRUITING



Computational Drug Repurposing for All EBS Cases

ClinicalTrials.gov ID
NCT03269474

The study will compare gene expression differences between blistered and non-blistered skin from individuals with all subtypes of EB, as well as normal skin from non-EB subjects. State of the art computational analysis will be performed to help identify new drugs that might help all EB wound healing and reduce pain.

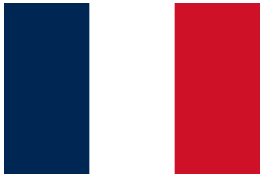
RECRUITING

Injections of Botulinic Toxin in Plantar Lesions of Localized Epidermolysis Bullosa Simplex (EBTox)

ClinicalTrials.gov ID
NCT03453632

The investigators hypothesize that palmar injections of botulinic toxin, via an inhibition of the sudation, would limit the occurrence of blisters in localized epidermolysis bullosa simplex (LEBS).

RECRUITING



Study to Evaluate Safety and Efficacy of ALLO-ASC-SHEET in Subjects With Dystrophic Epidermolysis Bullosa

ClinicalTrials.gov ID
NCT05157958

After confirming eligibility, a single subject with four selected target lesions will receive both ALLO-ASC-SHEET and Vehicle control, three target lesions for ALLO-ASC-SHEET and the other target for Vehicle control, and which lesion to apply which IP treatment will be determined randomly at the time of enrollment using pre-designed block randomization scheme.

RECRUITING

Long-Term Follow-up Protocol

ClinicalTrials.gov ID
NCT04917887

The main objective of this prospective, observational, long-term follow-up (LTFU) study is to evaluate the long-term safety profile of the gene therapy products evaluated by Krystal Biotech, Inc. which have a shared backbone of HSV-1, in participants who received at least one dose of investigational product (IP).

RECRUITING

Characteristics of Patients With Recessive Dystrophic Epidermolysis Bullosa

ClinicalTrials.gov ID
NCT01019148

Patients with RDEB develop large, severely painful blisters and open wounds from minor trauma to their skin. We are screening subjects with RDEB to evaluate characteristics of the subjects and their cells in order to develop new strategies of therapy and determine whether subjects could be candidates for treatment studies.

RECRUITING

Intravenous Gentamicin Therapy for Recessive Dystrophic Epidermolysis Bullosa (RDEB)

ClinicalTrials.gov ID
NCT03392909

Herein, the investigators propose evaluating the safety and efficacy of intravenous gentamicin in these patients. In theory, this intravenous administration has the possibility of treating simultaneously all of the patients' skin wounds. The milestones will be increased C7 and AFs in the patients' DEJ, improved EB Disease Activity Scores, and absence of gentamicin side effects.

RECRUITING

Gentamicin for Junctional Epidermolysis Bullosa

ClinicalTrials.gov ID
NCT03526159

Herein, the investigators propose the first clinical trial of gentamicin (by topical and intravenous administration) in JEB patients with nonsense mutations. The milestones will include restored laminin 332 and hemidesmosomes at the DEJ, improved wound closure, and the absence of significant gentamicin side effects.

RECRUITING

Optimizing IV Gentamicin in JEB

ClinicalTrials.gov ID
NCT04140786

The investigators propose to optimize the intravenous gentamicin regimen including dosage and infusion schedules to enhance the therapeutic outcome. The milestones will be an increase of laminin 332 in the patients' DEJ, improvement in EB Disease Activity Scores, and no gentamicin-associated side effects.

RECRUITING

Rigosertib for RDEB-SCC

ClinicalTrials.gov ID
NCT03786237

This project will evaluate whether rigosertib is capable of inducing an anti-cancer response in EB patients and whether the drug is well-tolerated. Mechanisms of molecular targeting of squamous cancer cells by rigosertib will further be investigated in EB patients, also aiming at the identification of biomarkers that may allow the predictive identification of best responders.

RECRUITING

Rigosertib in Patients With Recessive Dystrophic Epidermolysis Bullosa Associated SCC

ClinicalTrials.gov ID
NCT04177498

This pilot trial studies how rigsertib sodium works in treating patients with Recessive Dystrophic Epidermolysis bullosa (RDEB) with locally advanced Squamous Cell Carcinoma (SCC). Rigosertib may selectively target Epidermolysis bullosa (EB) cancer cells while leaving normal EB cells unaffected.

RECRUITING

Study of the Blood and Skin Immunological Profile of Patients With Recessive Dystrophic Epidermolysis Bullosa: in Vivo Analysis and the Impact of Placental Stem Cells in Vitro (ISTRADEB)

ClinicalTrials.gov ID
NCT06177353

Our primary objective is to define the systemic immunological/inflammatory signature of patients with RDEB with an aim to develop a strategy that involves using stem cells with high immunomodulatory/anti-inflammatory capacity such as allogeneic placental stem cells (WJ-MSCs and trophoblasts).

RECRUITING

An International, Multicenter, Randomized, Double-Blind, Parallel Group, Vehicle-Controlled, Phase 2/3 Study with Open-Label Extension Evaluating the Efficacy and Safety of Diacerein 1% Ointment for the Treatment of Generalized Epidermolysis Bullosa Simplex (EBS) (EBShield)

ClinicalTrials.gov ID
NCT06073132

The proposed Phase 2/3 trial with double-blind and open-label extension phases is an international, multicenter study designed to assess the efficacy and safety of diacerein 1% ointment in patients with generalized EBS.

RECRUITING

A Long-Term Extension Study for Participants Previously Treated With EB-101 for the Treatment of RDEB

ClinicalTrials.gov ID
NCT05708677

This is an open-label, long-term, follow-up study in participants from prior interventional trials involving surgical application of gene-corrected keratinocyte sheets (EB-101) for the treatment of RDEB wound sites.

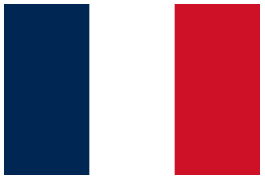
ENROLLING BY INVITATION

FARD (RaDiCo Cohort) (RaDiCo-FARD) (FARD)

ClinicalTrials.gov ID
NCT05954416

The goal of this observational study is to conduct a prospective assessment of the individual Burden of 9 rare skin diseases to assess disability in the broadest sense of the term (psychological, social, economic and physical) for patients and/or families.

RECRUITING



Reproductive Options in Inherited Skin Diseases (REPRO-ISD)

ClinicalTrials.gov ID
NCT06330324

The goal of this observational study is to learn about the indications for prenatal diagnostics and preimplantation genetic testing for patients/couples affected by an inherited skin disease and evaluate the clinical outcomes of these reproductive options.

ENROLLING BY INVITATION

Qualitative Study in Patients With Genodermatoses and Healthcare Professionals on Reproductive Counselling

ClinicalTrials.gov ID
NCT06330350

The investigators hypothesize that: a) intervention with dupilumab will improve itch in patients with pruritic genetic inflammatory skin disorders, even those not recognized to be Th2-driven; and b) the administration of dupilumab will be well-tolerated, regardless of underlying genetic skin disorder. The total clinical study duration will be 26 months (104 Weeks). The treatment period will include a 16-week open-label phase and a 20-month long-term extension phase for those who qualify and wish to continue.

RECRUITING

Repurposing Dupilumab for Management of Pruritic Genetic Inflammatory Skin Disorders

ClinicalTrials.gov ID
NCT05649098

The goal of this observational study is to conduct a prospective assessment of the individual Burden of 9 rare skin diseases to assess disability in the broadest sense of the term (psychological, social, economic and physical) for patients and/or families.

RECRUITING

Apart from these 24 trials, others are active but not yet recruiting

Clinical trials are a lot of work, but collectively what do we want to see?

GENE THERAPY

- VYJUVEK
- VYJUVEK EYE DROPS
- OTHER HSV DELIVERY
- SAME TECHNOLOGY FOR OTHER FORMS OF EB?

GENE THERAPY

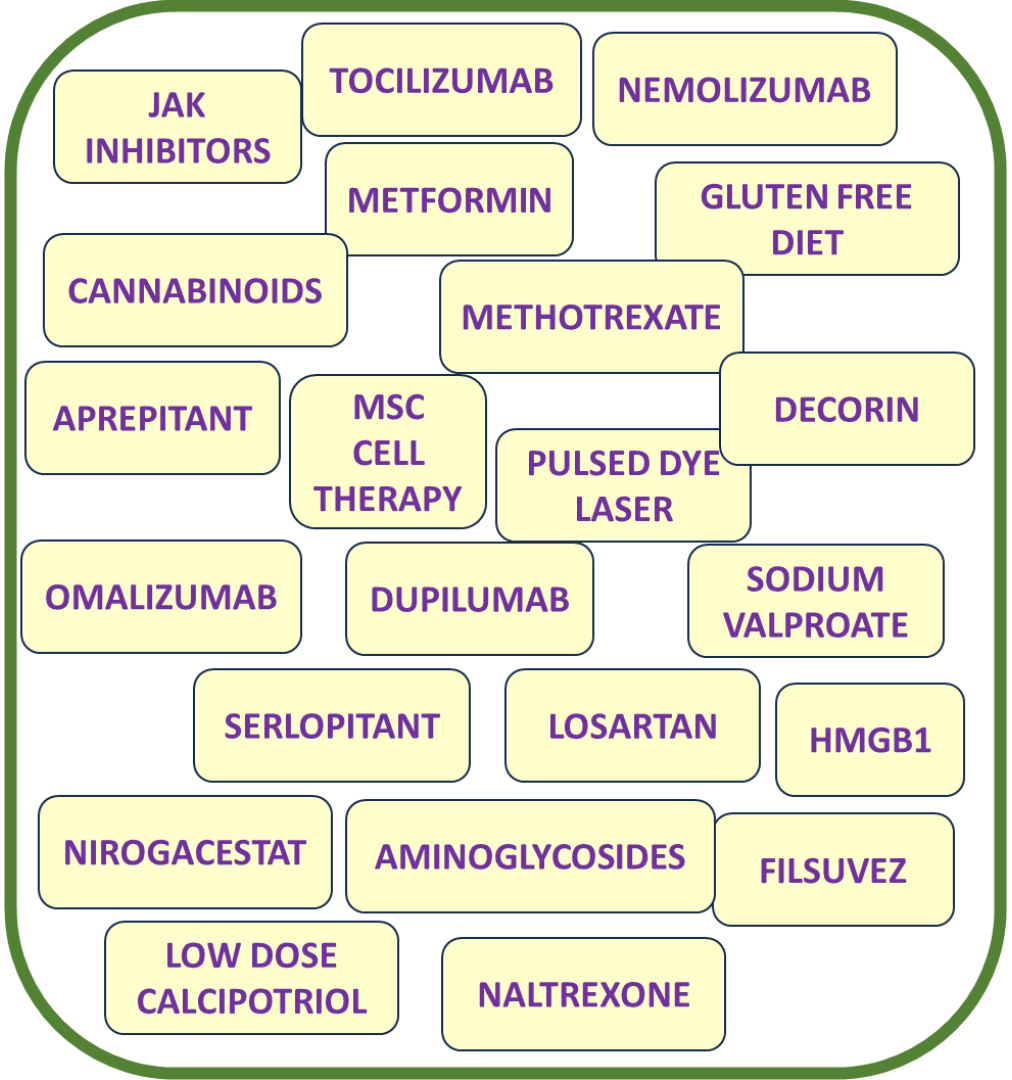
- HOLO-7, HOLO-X etc
- KERATINOCYTES?
- *EX VIVO* GRAFTING?
- EXON SKIPPING?
- GENE EDITING?

GENE THERAPY

THE CONTINUED FOCUS ON *COL7A1*?

TOPICAL OR SYSTEMIC?

DISEASE MODIFIERS



TRIAL DESIGN

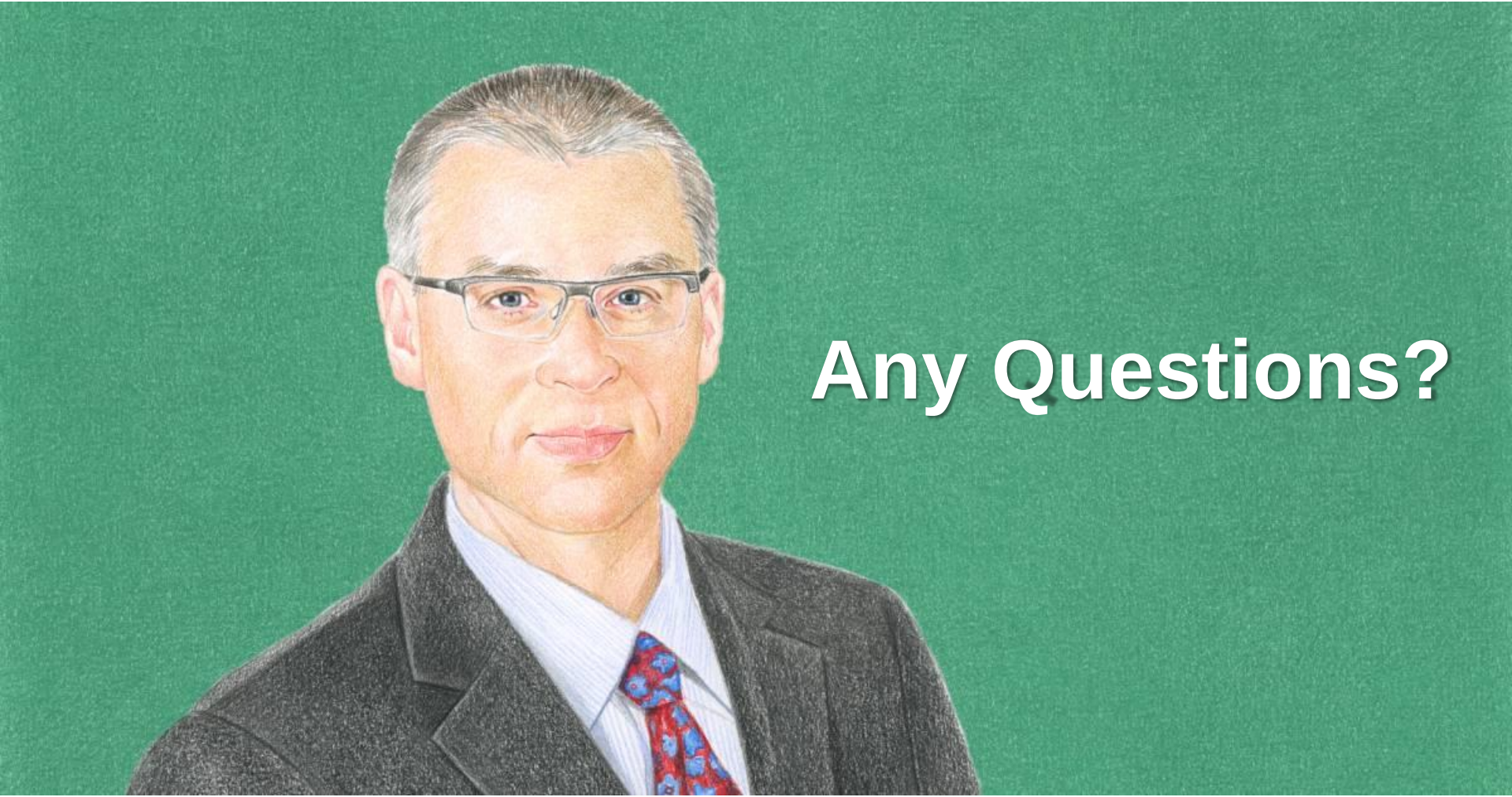
MEANINGFUL OUTCOMES

INCLUSION/ EXCLUSION

BEYOND PHASE 1/2

ENDPOINTS

PRODUCTS FOR PATIENTS


Any Questions?