NOW RECRUITING

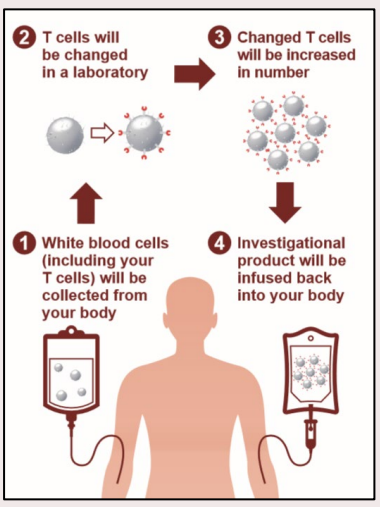
Cabaletta Bio™

DesCAARTes™ Phase 1 Trial

A clinical trial for patients with mucosal pemphigus vulgaris

WHAT IS MUSCOSAL PEMPHIGUS VULGARIS?

Mucosal pemphigus vulgaris (mPV) is an autoimmune disorder in which painful blisters are formed on the inside of the mouth, nose, throat, eyelids, anus, and genitals. In an autoimmune disorder, your body's own defense system (*the immune system*) attacks the body by mistake. In mPV, *B cells* – a type of immune cell in your body – mistakenly attack a protein related to skin cells, called *desmoglein 3* (DSG3). B cells are important for fighting infections, but "bad" B cells make antibodies that attack DSG3. This causes formation of painful blisters.



HOW IS mPV TREATED?

mPV can be treated with corticosteroids or immunosuppressant medications. However, these medications suppress the entire immune system. This puts people at risk of serious infections and other side effects. Therefore, new personalized treatment options are needed for people with mPV.

WHAT IS DSG3-CAART?

The investigational product, *DSG3-CAART*, will be made from your own T cells. Your white blood cells (including your T cells) will be collected from your body and your T cells will be changed (genetically modified) in a laboratory. After the investigational product has been made from your own T cells, it will be returned to you as an infusion (through a needle in your vein). The investigational product is designed to change your T cells, so they attack and kill the "bad" B cells in your body that cause pemphigus vulgaris.

CLINICAL TRIAL OVERVIEW

WHAT IS A CLINICAL TRIAL?

PRECLINICAL TESTING

Drug testing begins with extensive laboratory research in cells and animals that can last years. If successful, this data is sent to the Food & Drug Administration (FDA) for clearance to test in humans.

CLINICAL TRIALS

Purpose is to collect data about the safety and efficacy of an experimental drug. There are several steps in the clinical trials process before an experimental drug can be available to the public.

The drug development process includes 2 main stages: preclinical testing and clinical trials. DSG3-CAART has completed preclinical testing and is currently in the clinical trial stage, which is typically broken down into Phases 1, 2 and 3. During this stage, clinical research studies are conducted. A *clinical research study* is a medical study that helps to answer important questions about an investigational medication or product, such as: How safe is it? What is a safe amount, or dose? Does it work? What dose might work best?

All medications must be tested in clinical research studies before they can be approved to prescribe to patients. Without people taking part in these studies, we would have no new medications.

WHAT IS THE DesCAARTes™ PHASE 1 TRIAL?

This study is being done to find the highest dose of an investigational product (DSG3-CAART) that can safely be given to people with mPV. About 30 people with mPV are expected to take part.

Information on DesCAARTes™ trial site locations is available at ClinicalTrials.gov.

ELIGIBILITY CRITERIA

HOW DO I ENROLL IN THE TRIAL?

You may be able to take part if:

- You are 18 years of age or older
- You have been diagnosed with mPV
- You have active disease (at least 1 mucosal sore or blister)
- You have taken at least 1 medication for mPV that did not work well enough, had to be stopped because of side effects, and/or was not suitable for you

WHAT WILL THE DeSCAARTes™ STUDY INVOLVE?

If you take part, you may be in the study for up to 3.5 years and the Food and Drug Administration (FDA)-required long-term follow-up of 15 years to ensure your safety. The study is made up of 5 periods.

1 Screening
You will visit the study center for some tests or assessments to see if you are suitable for the study and whether you want to take part.

2 Pre-treatment
You will visit the study center up to 3 times for tests and assessments. During one of these visits, your T cells will be collected at a specialized clinic in order to make the investigational product.

3 Study treatment
You will visit the study center up to 5 times for study treatment and study assessments. You will receive the dose of the investigational product, split into up to 4 infusions.

4 Follow-up
You will visit the study center for study assessments to monitor your health. In the 1st month, you will visit the study center up to 6 times. After the first month, your visits will be monthly. After the 6th month, you will visit the study center every 3 months until Year 3.

5 Long-term
You will have a study visit every 6 months until Year 5 and once a year after that. These study visits may be done at the study center, by phone, or through email or mail.

CONTACT US

Are you wondering if this clinical trial is right for you? A representative from our Patient Recruitment Center would be glad to discuss the study with you. Alternatively, please email the Cabaletta Study Team. Study participation is voluntary. By contacting us, you are under no obligation to take part in the study.

Patient Recruitment Center*
Phone: +1-800-711-4906
Email: cabalettatrials@iqvia.com

Cabaletta Study Team
Email: clinicaltrials@cabalettabio.com

*Please include only your name and telephone number in the email. Someone from the recruitment center will contact you to discuss the study.