ZEVRA THERAPEUTICS

ACER-002

- Zevra Therapeutics is developing ACER-002 (celiprolol) tablets for the treatment of VEDS in patients with a *COL3A1* mutation to reduce the risk of arterial and other hollow organ clinical events. Celiprolol HCl (celiprolol) was originally approved in Europe in 1983 for the treatment of hypertension and for the prevention and treatment of angina. Celiprolol was well tolerated in patients with VEDS.
- Zevra Therapeutics is a rare disease company combining science, data, and patient needs to create transformational therapies for diseases with limited or no treatment options. Our mission is to bring life-changing therapeutics to people living with rare diseases.
- Zevra Therapeutics (formerly Acer Therapeutics) obtained a right of reference to Sanofi's CELECTOL™ (celiprolol HCl tablets), which was approved in Europe for the treatment of hypertension and angina.

The use of celiprolol in this trial is experimental, which means that it is not approved by the U.S. Food and Drug Administration (FDA) for the treatment of patients with *COL3A1*-positive Vascular Ehlers-Danlos Syndrome (VEDS) or any other indication. Participation is voluntary and you can leave the study at any time.

Scan Here

Information on the ongoing clinical trial can be found at the link below and how to participate: https://discoverceliprolol.com/ NCT05432466.

WHAT IS THE ACER-002 CLINICAL TRIAL?

To pre-qualify for this clinical study, you must meet the following criteria:

- Must be greater than or equal to 15 years old
- Willing to obtain a magnetic resonance angiogram (MRA) image
- During screening, must have a genetic test performed, via a saliva sample, to confirm the presence of the *COL3A1* mutation which will confirm a VEDS diagnosis; you will need a positive test result for Vascular Ehlers-Danlos Syndrome with a *COL3A1* gene mutation.
- Must be able and willing to discontinue use of beta blockers under the care of your primary physician
- Should not have had an arterial rupture or dissection, uterine rupture, and/or intestinal rupture within the last 6 months
- Must be willing to complete all study procedures

WHAT IS VASCULAR EHLERS-DANLOS SYNDROME (VEDS)?

- Vascular Ehlers-Danlos Syndrome, or VEDS, is a genetic disorder that affects the body's connective tissue. Connective tissue holds all the body's cells, organs, and tissue together.
- VEDS is caused by changes (or mutations) in the gene called *COL3A1* that tells the body how to make collagen III.
- Common symptoms include thin, translucent skin, easy bruising, fragile arteries, muscles, and internal organs.
- The exact number of affected people with VEDS is not known but may be approximately between 6,000 and 8,000 affected people in the U.S.[1]

Citation: [1]What is Vascular Ehlers-Danlos syndrome?. The VEDS Movement. (2023, January 20). https://thevedsmovement.org/veds/what-is-veds/

MRC-2400138 09/24