ongoing trials - trial from other registries

A randomised, double-blind, placebo controlled, two-part study to evaluate the efficacy, safety, tolerability and pharmacokinetics of a repeat dose of inhaled ETD001 in people with cystic fibrosis - Ongoing

Code: ISRCTN11798668 **Year:** 2024 **Date:** 45384 **Author:**

Study design (if review, criteria of inclusion for studies)

Interventional double blind randomized cross over placebo controlled trial (Safety, Efficacy)

Participants

All genders, 18 years of age, who fit one of the following criteria: Women of childbearing potential who are willing and able to use contraception from a minimum of 28 days before receipt of the first dose of study medication until completion of the final follow up visit. Women of non-childbearing potential defined as being amenorrhoeic for >12 months with an appropriate clinical profile (e.g. age appropriate, menopausal symptoms). However, if indicated, this should be confirmed by follicle-stimulating hormone levels consistent with menopause (according to local laboratory ranges). Alternatively, women without a uterus or who have been permanently sterilised (e.g. hysterectomy, bilateral salpingectomy or bilateral oophorectomy, but not tubal ligation). Men who are willing and able to use one of the contraception methods from the time of the first dose, until completion of the final follow up visit. FEV1 = 40% and = 90% of predicted normal for age, gender, and height using Global Lung Function Initiative (GLI) standards. Be able to reproducibly perform spirometry manoeuvres. Clinically stable CF lung disease (no decrease in FEV1 > 10%, or signs and symptoms of acute pulmonary exacerbation such as: increased cough, change in sputum (volume or consistency), change in respiratory examination and respiratory rate, decreased appetite or weight loss, chest pain, hemoptysis, decreased lung function, fever defined as temperature > 38°C (100.4°F) within 28 days prior to Visit 1.

Interventions

Part A (safety and tolerability in participants with cystic fibrosis): 8 participants will receive 13 doses of ETD001 4.5 mg or placebo as an oral inhalation twice a day over 7 days, twice daily doses on Days 1 to 6 and a single dose on the morning of Day 7. 6 participants will receive active ETD001 and 2 will receive placebo. Allocation of participants to treatment type will be coordinated using a centralised interactive response technology (IRT) system. Part B (safety and efficacy in participants with cystic fibrosis): 22 participants will be required to complete two 28 day treatment periods, twice daily doses of ETD001 4.5 mg will be administered in one treatment period and twice daily doses of placebo will be administered in the other treatment period. In each treatment period a total 55 doses of ETD001 4.5 mg or placebo will be administered as an oral inhalation over 28 days, twice daily doses on Days 1 to 27 and a single morning dose on the morning of Day 28.

Outcome measures

1. Number of participants (n of P) reporting adverse events (AE) â€“ baseline to follow up (approximately 28 days) 2. n of P who discontinue due to an AE â€“ baseline to follow up (approximately 28 days) 3. n of P with vital sign abnormalities as assessed by measuring systolic and diastolic blood pressure, heart rate, respiration rate, temperature and peripheral oxygen saturation â€“ baseline to follow up (approximately 28 days) 4. n of P with spirometry abnormalities as measured using a spirometer â€“ baseline to follow up (approximately 28 days) 5. n of P with laboratory test abnormalities measured using biological samples â€“ baseline to follow up (approximately 28 days) 6. n of P with electrocardiogram (ECG) abnormalities measured using 12-Lead ECG recordings â€“ baseline to follow up (approximately 28 days)

https://www.isrctn.com/ISRCTN11798668

Keywords

pharmacological_intervention; ENaC antagonists - Sodium Channel Blockers; Amiloride; Respiratory System Agents; EDT001;