Vertex Trial VX-445-102

Phase: Phase 3
Treatment: Triple combination modulators (Ivacaftor, Tezacaftor and Elexacaftor)
Population: Patients with CF whose genotype included one copy of the Phe508del and a minimally function gene
Age: 12 +
Duration: 24 weeks

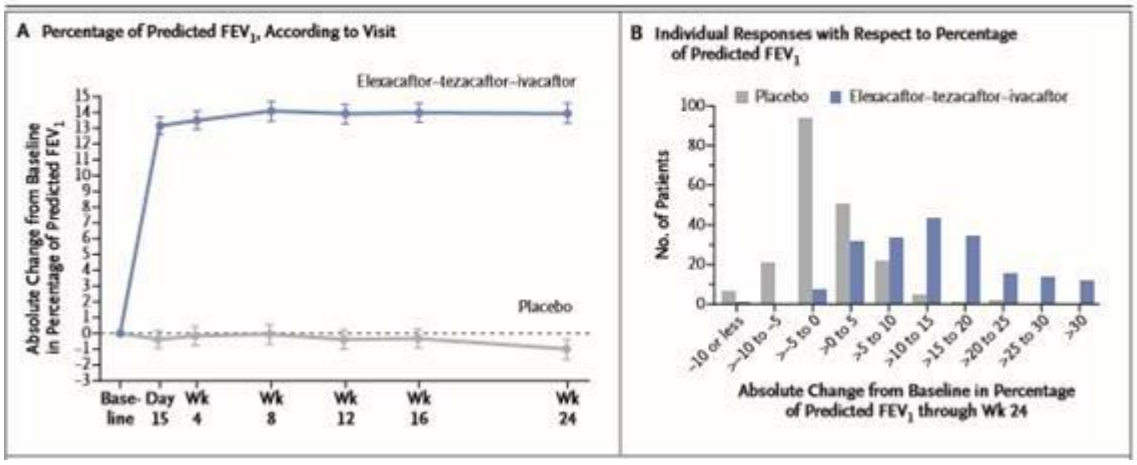
Results:

403 patients across the globe were randomised to receive either the triple combination drug (study drug) or a placebo drug for 24 weeks. The outcomes to be assessed between the groups were: lung function, (primarily change in percent predicted for Forced Expiratory Volume in one second (FEV_1)); rate of respiratory exacerbations; and, the respiratory score from the Cystic Fibrosis Quality of health measurement (CFQR).

At the end of the 24 week study the group who received the study drug had a 14.3% increase in FEV_1 compared to the placebo group. There were a range of individual responses to the drug from no change in FEV_1 to more than a 30% change in FEV_1. The rate of respiratory exacerbations was 63% lower in the study drug group compared to the placebo group. Patients reported a 20 point higher quality of life score in the CFQR (4 point difference is significant). Generally the side effects were mild to moderate, with only 2 patients in the study drug group discontinuing the study due to adverse events.

Since this trial was published in the New England Journal of Medicine, Elexacaftor, Ivacaftor, and Tezacaftor (Brand name Trikafta) has now been approved by the FDA. The drug still needs approval by Health Canada and other regulatory agencies before being available to Canadians with CF.

The NEW ENGLAND JOURNAL of MEDICINE



https://www.nejm.org/doi/full/10.1056/NEJMoa1908639