

primary studies - published, non RCT

Challenged Urine Bicarbonate Excretion as a Measure of Cystic Fibrosis Transmembrane Conductance Regulator Function in Cystic Fibrosis.

Code: PM36315944

Year: 2022 **Date:**

Author: Berg P

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

Prospective observational study.

Participants

50 adult patients with CF starting CFTR modulator therapy with elexacaftor/tezacaftor/ivacaftor between May 2020 and June 2021. Cystic fibrosis clinic, Aarhus University Hospital, Denmark.

Interventions

elexacaftor/tezacaftor/ivacaftor

Outcome measures

Quantification of urine bicarbonate excretion after an acute oral sodium bicarbonate challenge before and 6 months after elexacaftor/tezacaftor/ivacaftor treatment.

Main results

At baseline, challenged urine bicarbonate excretion was associated with several CF disease characteristics. Bicarbonate excretion was higher in patients with residual function mutations. A higher bicarbonate excretion was associated with better lung function, pancreatic sufficiency, and lower relative risk for chronic pseudomonas infections. Elexacaftor/tezacaftor/ivacaftor treatment increased bicarbonate excretion by 3.9 mmol/3 h (95% CI, 1.6 to 6.1 mmol/3 h), reaching about 70% of that seen in healthy control participants. In healthy control participants, individual bicarbonate excretion at each visit correlated with the individual mean bicarbonate excretion. The median coefficient of variation was 31%.

Authors' conclusions

Although further studies are needed to address the performance and sensitivity of this approach, this early-stage evaluation shows that challenged urine bicarbonate excretion may offer a new, simple, and safe quantification of CFTR function and the extent of its pharmacologic improvement. Elexacaftor/tezacaftor/ivacaftor partially restores renal CFTR function in patients with CF, likely resulting in decreased risk for electrolyte disorders and metabolic alkalosis.

<http://dx.doi.org/10.7326/M22-1741>

See also

Ann Intern Med. 2022 Nov;175(11):1543-1551. doi: 10.7326/M22-1741. Epub 2022 Nov 1.

Keywords

Adult; Aged; CFTR Modulators; Genetic Predisposition to Disease; pharmacological_intervention; placebo; VX-770; VX-661; ivacaftor; Aminophenols; tezacaftor; VX-445; elexacaftor; non pharmacological intervention - diagn; Trikafta;