primary studies - published RCT

Efficacy and safety of the elexacaftor plus tezacaftor plus ivacaftor combination regimen in people with cystic fibrosis homozygous for the F508del mutation: a double-blind, randomised, phase 3 trial.

Code: PM31679946 **Year:** 2019 **Date:** **Author:** Heijerman HGM

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

Randomized controlled trial (RCT) involving 19 CF centres

Participants

Adults with Cystic Fibrosis (CF) by supporting adherence to nebuliser medication. 19 CF centres, with 32 interventionists, 305 participants in the intervention group, and 303 participants in the standard care arm.

Interventions

A multi-component self-management intervention 'CFHealthHub' was developed to reduce pulmonary exacerbations in adults with Cystic Fibrosis (CF) by supporting adherence to nebuliser medication. Interventionists underwent training and competency assessments to be deemed certified to deliver the intervention.

Outcome measures

Fidelity of the CFHealthHub intervention and standard care was assessed using different methods for each of the five fidelity domains defined by the Borrelli framework: study design, training, treatment delivery, receipt, and enactment. Study design ensured that the groups received the intended intervention or standard care. Interventionists underwent training and competency assessments to be deemed certified to deliver the intervention. Audio-recorded intervention sessions were assessed for fidelity drift. Receipt was assessed by identifying whether participants set Action and Coping Plans, while enactment was assessed using click analytics on the CFHealthHub digital platform.

Main results

Design: There was reasonable agreement (74%, 226/305) between the expected versus actual intervention dose received by participants in the CFHealthHub intervention group. The standard care group did not include focused adherence support for most centres and participants. Training: All interventionists were trained. Treatment delivery: The trial demonstrated good fidelity (overall fidelity by centre ranged from 79 to 97%), with only one centre falling below the mean threshold (>â€‰80%) on fidelity drift assessments. Receipt: Among participants who completed the 12-month intervention, 77% (205/265) completed at least one action plan, and 60% (160/265) completed at least one coping plan. Enactment: 88% (268/305) of participants used web/app click analytics outside the intervention sessions. The mean (SD) number of web/app click analytics per participant was 31.2 (58.9). Additionally, 64% (195/305) of participants agreed to receive notifications via the mobile application, with an average of 53.6 (14.9) notifications per participant.

Authors' conclusions

CONCLUSIONS: The study demonstrates high fidelity throughout the RCT, and the CFHealthHub intervention was delivered as intended. This provides confidence that the results of the RCT are a valid reflection of the effectiveness of the CFHealthHub intervention compared to standard care.

http://dx.doi.org/10.1016/S0140-6736(19)32597-8

See also

Lancet. 2019 Oct 30. pii: S0140-6736(19)32597-8. doi: 10.1016/S0140-6736(19)32597-8.

Keywords

Self-Management; Organization; non pharmacological intervention - psyco-soc-edu-org; Inhalation OR nebulised; nebuliser; Behavioural

interventions;