

primary studies - published RCT

GLPG2737 in lumacaftor/ivacaftor-treated CF subjects homozygous for the F508del mutation: A randomized phase 2A trial (PELICAN).

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

Randomized controlled trial

Participants

82 patients with self-reported CF.

Interventions

Participants were randomized to either the MukoHelp group (n = 41) or a waitlist control group (n = 41).

Outcome measures

Patients were assessed before (baseline), after the six-week intervention period (post), and three months after post (follow-up). Assessments included acceptability (Client Satisfaction Questionnaire; CSQ), efficacy (e.g., anxiety; Generalized Anxiety Disorder-7 Scale; GAD-7), and adherence.

Main results

MukoHelp received mixed acceptability with a high acceptance according to the CSQ (75.7%), but adherence was low, indicating low acceptability. The intervention did not lead to significant improvements in symptoms of anxiety compared to waitlist controls ($p = .645$, $\hat{I}(p) = 0.005$). Exploratory analyses suggest that low adherence impacted efficacy as the item "I apply what I have learned in the MukoHelp chapters in my everyday life" was negatively correlated at a moderate effect size with the GAD-7 baseline-post difference score ($r = -0.500$, $p = .035$).

Authors' conclusions

While some participants reported satisfaction, overall engagement and adherence were low, and no symptom improvements were observed. This suggests that future versions of MukoHelp should focus on increasing sustained usage-potentially through added guidance or supportive elements-to enhance feasibility and therapeutic impact.

<http://dx.doi.org/10.1016/j.jcf.2019.09.006>

See also

J Cyst Fibros. 2020 Mar;19(2):292-298. doi: 10.1016/j.jcf.2019.09.006. Epub 2019 Oct 5.

Keywords

Adolescent; Adult; Caregivers; non pharmacological intervention - psycho-soc-edu-org; Self-Management; Behavioural interventions; Psychoeducation; training; Depression; Mental Disease-Psychiatric Conditions;