

REAL-LIFE SAFETY AND SATISFACTION OF CFTR PROTEIN MODULATORS IN CYSTIC FIBROSIS

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BACKGROUND

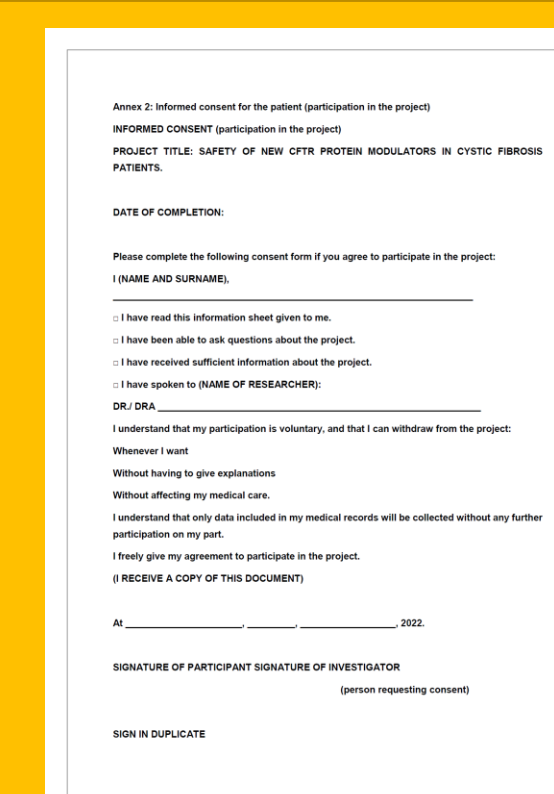
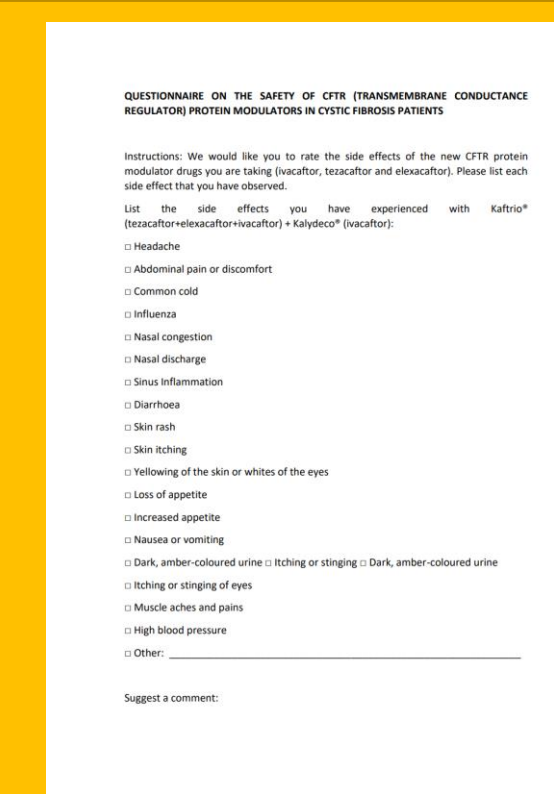
The new transmembrane conductance regulator (CFTR) modulator drugs (ivacaftor/tezacaftor/elexacaftor) are bringing about a major change in the treatment and quality of life of cystic fibrosis (CF) patients. There is a need to collect information on patient perception of benefit and safety.

OBJECTIVE

To assess the satisfaction and adverse effects (AE) profile reported by patients on ivacaftor/tezacaftor/elexacaftor treatment.

MATERIAL AND METHODS

- Observational, prospective, single-centre study from March to June 2022.
- Variables: sociodemographic (age and sex) and biochemical (GOT-ASAT, GPT-ALAT, bilirubin and CPK) collected from patients' medical records.
- Treatment Satisfaction Questionnaire for Medication (TSQM 1.4): with 14 items in four scales.
- Adverse Effects Questionnaire (ad hoc).
- Patient Informed Consent.



RESULTS

- 58 patients, 43 answered the questionnaires, 17 (40%) female, median age 30 (26-37)

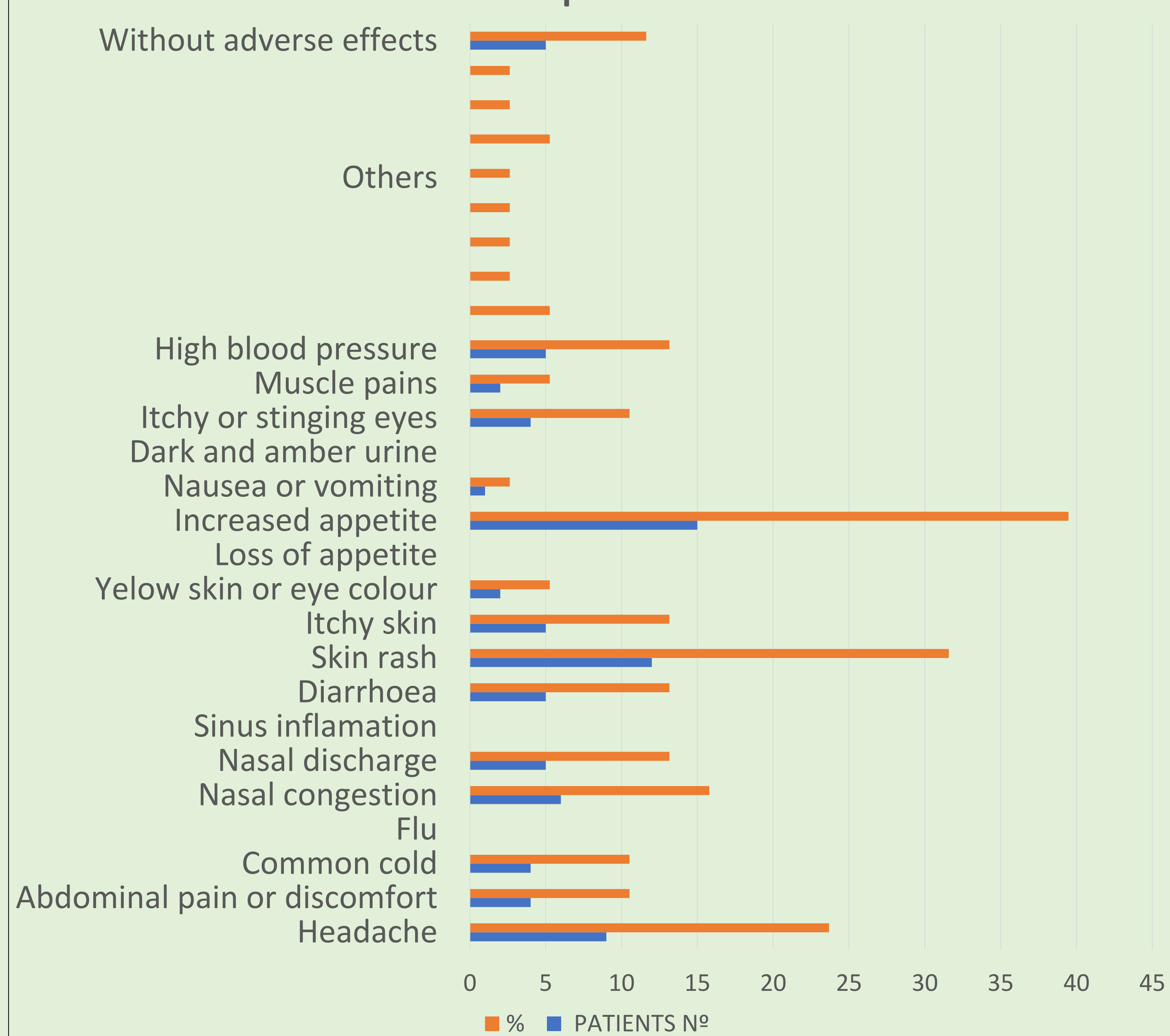
TSQM 1.4 questionnaire results

	Question	Average score	Maximum posible score
Efficacy	1-3	21 (20-21)	21
Adverse effects	4-8	19 (16-20)	21
Convenience	9-11	21 (18-21)	21
Overall satisfaccion	12-14	17 (16-17)	17

Biochemical variables three months after starting treatment

- three patients had GPT-ASAT >3 LSN and only one patient >5 LSN
- one patient had increased CPK >5 LSN
- no patient had bilirubin >2 LSN

Adverse effects questionnaire results



Other adverse effects:

- Muscle spasms
- Acne
- Memory loss
- Stomach pain
- Insomnia
- Increased bilirubin
- Fluid retention
- Flatulence

CONCLUSIONS

Although a high percentage of patients have experienced AEs, CFTR modulators are widely accepted drugs with a favourable AE profile. The most frequent AEs reported by patients were increased appetite, rash and headache. The AEs described by patients are described in the data sheet. More real-life studies are needed to confirm our study and to provide further evidence.

