Conclusions: These seven patients with N1303K and a second non-responsive mutation demonstrated significant clinical improvement after treatment with ELX/TEZ/IVA. A controlled clinical trial is needed to confirm these results and allow people with the N1303K mutation to register and be reimbursed.

References
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Background: People with cystic fibrosis (CF) have recurrent respiratory tract infections and acute pulmonary exacerbations (PEx), which cause loss of lung function and thus decrease life expectancy. One sign of PEx is decline in forced expiratory volume in 1 second (FEV₁). Early detection of impairment in pulmonary function tests (PFTs) allows early diagnosis and treatment of PEx. We aimed to investigate the effect of electronic home spirometry in people with CF on number of PEx and 1-year change in percentage predicted FEV₁.

Methods: This was a randomized, 1-year, prospective study. Children with CF aged 6 to 18 who could perform spirometry and had a smartphone were included. Eligible children were randomized into two groups: home spirometry (HSG) and usual care (UCG). HSG participants performed two PFTs per week, and a registered CF nurse evaluated the results for decline in FEV₁; simultaneously. Number of PEx, days in hospital for PEx, and days on oral antibiotics for PEx were evaluated from patients’ records and compared with data from the previous year. A health-related quality of life (QOL) questionnaire for children with CF was administered and lung clearance index (LCI) was measured at the beginning and end of the study.

Results: Each group included 30 patients; 22 in the HSG and 24 in the UCG completed the study. Median age was 13.5 (IQR 11.0–14.9) in the HSG and 12.7 (IQR 10.6–15.5) in the UCG. Median 1-year change in FEV₁/pp was 0.95 (IQR –2.61–6.73) in the HSG and –0.41 (IQR –3.58–3.09) in the UCG (p = 0.27). The results are summarized in Table 1.

Conclusions: Electronic home monitoring of children with CF using spirometry and early treatment of PEx may result in slower decline in lung function, which would be likely to have a beneficial effect on QOL. Full results, including QOL analysis, will be available before the conference.