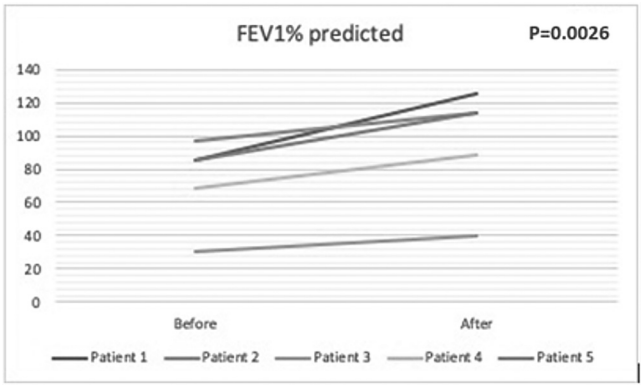

Figure 1. Values before and 4 to 8 weeks after starting treatment with elexacaftor/tezacaftor/ivacaftor

Conclusions: These seven patients with N1303K and a second nonresponsive 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.

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134

Effect of hypertonic saline therapy on lung clearance index in preschool children with cystic fibrosis

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Background: Hypertonic saline (HS) may improve mucociliary clearance in the context of long-term maintenance therapy in people with cystic fibrosis (CF) aged 7 and older. We aimed to assess the effect of inhaled HS on the lung clearance index ($LCI_{2.5}$) and percentage predicted forced expiratory volume in 1 second (FEV_1pp) in children aged 4 to 6 with CF.
Methods: The study was conducted at the Division of Pediatric Pulmonology, Marmara University, between May and October 2021. Participants, who had a confirmed diagnosis of CF between the ages of 48 and 72 months, were divided into two groups as 7% HS users and 0·9% isotonic saline (IS) users. $LCI_{2.5}$ was measured according to nitrogen multiple-breath washout, FEV_1pp were measured, and the Pediatric Quality of Life Inventory (PedsQL) was administered at baseline and week 24.
Results: There were 16 children each in the HS and IS groups. All were on dornase alpha treatment. Cough was reported as an adverse event in nine children in the HS group. Mean $LCI_{2.5}$ was 9.4 ± 3.1 at baseline and 8.0 ± 1.9 at week 24 in the HS group ($p = 0.01$) and 8.2 ± 1.6 at baseline and 7.8 ± 2.1 at week 24 in the IS group ($p = 0.42$). There was no significant change in FEV_1pp between baseline at week 24 in either group ($p > 0.05$). Scores on the physical domain of the PedsQL in the IS group increased between baseline and week 24 ($p = 0.04$).
Conclusions: In this group of preschool-aged children with CF, treatment with HS for 24 weeks was associated with significantly greater improvement in $LCI_{2.5}$ than treatment with IS.

135

Electronic home monitoring of children with cystic fibrosis to detect and treat acute pulmonary exacerbations and its effect on 1-year FEV_1 loss

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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 (FEV_1). 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 FEV_1pp.
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 FEV_1 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 (interquartile range (IQR) 11.7–14.9) in the HSG and 12.7 (IQR 10.6–15.5) in the UCG. Median 1-year change in FEV_1pp was 0.95 (IQR −2.61–6.73) in the HSG and −0.41 (IQR −3.68–3.09) 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.

136

Long-term clinical impact of a virtual model of care in cystic fibrosis

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Background: Health professionals have explored and tested the use of digital technologies provide in managing chronic respiratory diseases remotely during the COVID-19 pandemic. Further investigation into whether these new approaches to care delivery provide an opportunity to improve cystic fibrosis (CF) management is needed. The aim of this study was to assess the long-term clinical impact of use of e-health as part of a virtual model of care in CF.

Table 1 (abstract 135):
Comparison of home spirometry and usual care group results

	Home Spirometry Group	Usual Care Group	p
Median FEV_1 pp in the previous year (median, 25-75.p)	95 (87-107)	94 (87-109)	0.71
Median FEV_1 pp during the study period (median, 25-75.p)	101 (89-107)	94 (87-107)	0.44
Change in FEV_1 pp (median, 25-75.p)	0.95 (-2.61- 6.73)	-0.41 (-3.68-3.09)	0.27
Median FEV_1 z score in the previous year (median, 25-75.p)	-0.24 (-0.70-0.46)	-0.39 (-0.79-0.55)	0.49
Median FEV_1 z score during the study period (median, 25-75.p)	0.10 (-0.70-0.44)	-0.36 (-0.97-0.50)	0.40
LCI 2.5 in the previous year (median, 25-75.p)	11.29 (7.98-14.84)	10.02 (7.60-13.37)	0.48
LCI 2.5 during the study period (median, 25-75.p)	9.32 (7.37-12.37)	8.59 (6.79-11.96)	0.50
Number of total exacerbations in the previous year (median, 25-75.p)	2 (1-3)	1 (0-4)	0.41
Number of total exacerbations during the study period (median, 25-75.p)	3 (1-4)	1 (0-2)	**0.02**
Number of hospitalizations in the previous year (median, 25-75.p)	0 (0-1)	0 (0-1)	0.58
Number of hospitalizations during the study period (median, 25-75.p)	0 (0-1)	0 (0-0)	**0.04**
Number of days in hospital in the previous year (median, 25-75.p)	0 (0-13)	0 (0-25)	0.51
Number of days in hospital during the study period (median, 25-75.p)	0 (0-13)	0 (0-0)	**0.04**
Number of oral antibiotics in the previous year (median, 25-75.p)	2 (1-3)	1 (0-2)	0.19
Number of oral antibiotics during the study period (median, 25-75.p)	2 (1-4)	1(0-2)	**0.04**
Number of days on oral antibiotics in the previous year (median, 25-75.p)	28 (14-46)	14 (4-28)	0.19
Number of days on oral antibiotics during the study period (median, 25-75.p)	28 (14-56)	14 (0-28)	**0.04**

Methods: The NuvoAir Home platform consists of a smartphone application, Bluetooth spirometer, and clinician portal. Patients were trained to use the platform and asked to perform home spirometry monthly. Data on pulmonary function and pulmonary exacerbations were collected at baseline and after 12 months. A survey was emailed to evaluate patients' experience using the technology.

Results: A cohort of 43 people with CF were recruited (26 female; mean age 31.6 ± 6.8; 16 homozygous for delta F508; FEV_1 48.4 ± 16.3% predicted). Sustained improvement in forced expiratory volume in 1 second (FEV_1) expressed as absolute and percentage predicted was seen through 12 months (mean absolute change 100 mL, $p = 0.02$; mean percentage predicted change 3.8%, $p = 0.005$). We found significant improvement in forced vital capacity expressed as absolute value (mean change 230 mL, $p = 0.006$) and percentage predicted (mean change 6.2%, $p = 0.002$). The average of number of exacerbations per person 1 year before use of digital technology was 0.84, vs 0.09 1 year after ($p < 0.001$). Ninety percent of patients reported that they understood their CF better after starting the virtual care service. No changes in medical treatment were reported during that time.

Conclusions: Use of digital technologies in the management of adults with CF improved lung function and decreased pulmonary exacerbations. People with CF readily accepted using a virtual model of care and improved their understanding of their medical condition.