HOME REPORTED OUTCOMES (HERO) STUDY: A REAL-WORLD RESEARCH PILOT OF VIRTUAL ENROLLMENT & HOME-TRACKING IN CF

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INTRODUCTION

The HomE-Reported Outcomes (HERO) Study was designed to assess the feasibility of large-scale collection and use of home-reported outcomes to answer high-impact questions in cystic fibrosis, at both the individual and population levels. As CFTR modulators are used by increasing numbers of CF patients, our ability to track impact on health and well-being, as well as to understand the most-effective combination of treatments for each individual patient, is limited without systematically collecting patient- and family-reported data.

This study particularly focused on the use of the Folia home-reported outcomes (HRO) capture and analysis platform by individual patients and caregivers. Folia is a web- and iOS-based application that is available free-of-charge to individual patients and families and has previously been piloted in cystic fibrosis. Centers participating in previous Folia pilots or the current Bridge program include Maine Medical Center, Dell Medical Center, University of Vermont Medical Center, and Keck Medical Center of USC.

AIMS

1. Understand the willingness of patients & families to consent to their data being used in research studies
2. Assess the consistency of data entry throughout the study
3. See an example of data to understand what treatments patients are using and how use varies over time
4. Assess the feasibility of linking data entered into Folia with data provided in the CF Foundation Patient Registry

METHODS

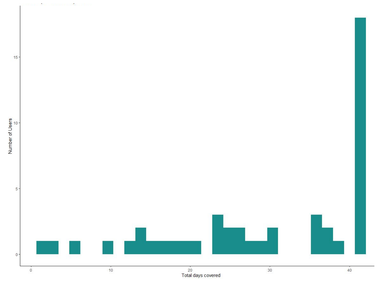
1. **Recruitment**
   a. To register a cohort of patients not yet using Folia, we proposed to use a geo-specific, bi-modal registration model, based on the distribution method that was shown to work effectively in Folia's three cystic fibrosis pilot programs which relies upon registration support from (1) the local clinics and (2) local cystic fibrosis advocates ("ambassadors").
2. **Study Preparation**
   a. In order to prepare existing Folia users for requests to enroll in the HERO study, the Folia team sent out 2 preliminary informational notes to all users in the CF community. These notes did not describe the details of the HERO study, focusing instead on the intention to begin a CF-focused research study on the Folia platform, and describing how to find research sharing settings on Folia.
3. **Registration and enrollment**
   a. Study registration and enrollment took place through the Folia Health app, with a dedicated research portal, which provided information about the study, a consent form, and study requirements.
4. **Data Collection**
   a. For this study, data was collected from the participants' responses to Folia Questions over a period of six weeks. Participants were asked to track their CF specific treatments, like Airway Clearance, and any relevant symptoms they experienced.

RESULTS

Symptom	Symptom group	Participants who tracked at least once	Total tracks	Tracks/ participant/ week (weighted)[1]	Tracks/ participant/ week (unweighted)[2]
Coughing	Respiratory	35	879	5.0	4.3
Bowel movement characteristics	Digestive	23	365	6.9	2.6
Bowel movement number	Digestive	14	357	4.9	4.3
Postnasal drip	Ear, Nose, & Throat	11	217	4.1	3.3
Appetite loss	Digestive	5	91	4.8	3
Stomachache (tummy ache)	Digestive	5	33	2.4	1.1

Group	Treatment	Participants tracking this treatment at all during study[1]	Treatment added to schedule after study start	Treatment turned off during study
Airway Clearance	Vest treatment	39	0	0
	Albuterol Sulfate	18	1	1
	Chest physical therapy (CPT)	10	1	2
	FLOVENT	7	0	0
	PROAIR HFA	6	0	0
	ADVAIR HFA	5	0	0
	VENTOLIN	4	0	0
	Budesonide or SYMBICORT	3	0	0
	Levalbuterol	2	1	0
	SINGULAIR	2	0	1
	Spiriva Respimat	2	0	0
	Huff Cough	1	0	0
	PULMICORT RESPULES	1	1	0
	Proventil HFA	1	0	0
	Xopenex HFA	1	0	0
Dornase Alfa	Pulmozyme	38	0	0
Hypertonic Saline	Hypertonic saline solution (7%)	24	1	0
	Hypertonic saline solution (3%)	10	0	2
	Hypertonic saline solution (2.25%)	4	0	1
Tobramycin	Tobramycin	6	3	0
Aztreonam	Aztreonam (Cayston) 75 mg/ml solution	6	0	1
Azithromycin	Azithromycin	12	3	0
CFTR Modulator	SYMDEKO	12	1	3
	Kalydeco	8	0	0
	Trikafta (elexacaftor 100 mg, tezacaftor 50 mg and ivacaftor 75 mg)	5	4	0
	ORKAMBI	2	1	0
	Trikafta (ivacaftor 150 mg)[2]	2	2	1

Number of weeks tracked	Study participant count
0	16
1	2
2	1
3	3
4	4
5	1
6	36



CONCLUSIONS

1. **Registration and enrollment met targets for this study (N=63), and there was strong expressed PCG interest in establishing understanding of experience of care and outcomes in-between clinic visits.** This interest seemed directed both at improving clinical understanding of family experience at home, and at developing research that incorporates these datapoints.
2. **Overall, participants were very consistent in data entry.** Among those who tracked, 77% tracked all 6 weeks of the study, and 34% tracked all 42 days. Over 6 weeks, we collected substantially more data on patient experiences than typical clinical trial data sets. Participants tracked hundred home reported observations on treatment use, symptoms, notes, and measurements.
3. **Over the six weeks of the study, participants tracked 24,630 observations, including 20,313 treatments and 3,150 symptoms.** The most commonly tracked treatments were those in the airway clearance group, dornase alfa, and hypertonic saline, and the symptoms tracked by the greatest number of participants were coughing, bowel movement (characteristics & frequency), postnasal drip, appetite loss, and stomachache. This is in line with expectations for the cystic fibrosis population.
4. **When collecting home-reported outcomes, patients and caregivers require a highly robust system to capture all of their observations.** Participants collected information on a wide variety of datatypes, with a significant long tail. The flexibility of the Folia tracking system allowed PCG to collect this diverse data in a structured way, without losing the ability to track less-common items, like bloody nose.
5. **The CFF Registry and Folia Research teams are working together** in order to integrate the Folia-collected home-reported outcomes data with CFF Registry data collected on each individual patient enrolled in this study.

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