ECFS 2024

S Bui

Psychologie

Moderniser l'évaluation et les soins psychosociaux dans le contexte des modulateurs de CFTR

ARISE ACHIVING ROUTINE SCREEN FOR EMOTIONAL HEALTH :

= recueil systématique des émotions (anxiété, dépression…) via un questionnaire électronique

- L'IDE envoie le questionnaire avant la prochaine visite si le patient oublie, il peut le remplir sur une tablette avant la CS
- 81% complétude donc bonne faisabilité
- 47% parents sont en détresse
- 20% tb sévères du sommeil chez les parents++

TROUBLES DU SOMMEIL ET ETI

AUGMENTATION DE LA DÉPRESSION ANXIÉTÉ MAIS QUID DU SOMMEIL CHEZ LES PETITS ?

- ETI mai 2023
- Etude prospective monocentrique dans le Queensland : 450 patients
- Questionnaires électroniques : T0 M1 M12 parents/enfants
- PSC17 141 enfants Age 8.6
- 75 ont complété les 1 mois
- Pas de différence significative
- Les troubles de l'attention à la baseline sont plus élevés
- Pour le sommeil : 10 patients ont augmenté les scores au dessus de la normale
- Troubles du comportements le 1er mois mais résolutifs
- Pas d'arrêt

PARENT AVEC LA MUCO

- 1/20 in UK ont un parent qui est décédé
- But : Enfant avec un parent qui a la muco peu d'étude regardent la vision de ces enfants.
- 8 participants 6-16 ans
- La compréhension de la maladie
  - en fonction de l'âge de compréhension
  - Quand ils font parti des soins
- S'inquiète pour le parent, sentiment d'impuissance feeling out of control
- Se sente responsable des soins et de la maladie
- Tristesse quand ils ne sont pas là ; inquiétude du DC
- Sentiment d'être différent et d'avoir une famille différente
- tristesse manque du parent…

GEMS-CF: Questionnaire pour les adultes

Cohorte très diverse en âge sexe race LGBTQ+

- Anxiété 90%/; Tb sommeil 90%
- Image négative du corps 85% (homme qui se trouve petit/ aux autres…)
- trauma PTSD 80% abus y compris abus sexuel ? pas d'automie du corps depuis longtemps= peu de limite + abus..

■Dg des problèmes psychologiques peu fait

Effets modulateurs 77%

- Idée suivcidaire 38%
- tb comportements alimentaire 37%, substance misuse de médicaments ou alcool
- Colère 32%

TOP des impacts des modulateurs

- Peur du futur 47% Image corps 42%
- Insomnie sommeil+ agitation : 33%

CALM Coping and learning to manage stress with CF : questionnaires/visio/entretien ?

- but diminuer 1er Depression ; anxiété ;
- adulte avec SC modérés pas d'idée suicidaire majeure

Comparaison CALM suivi classique

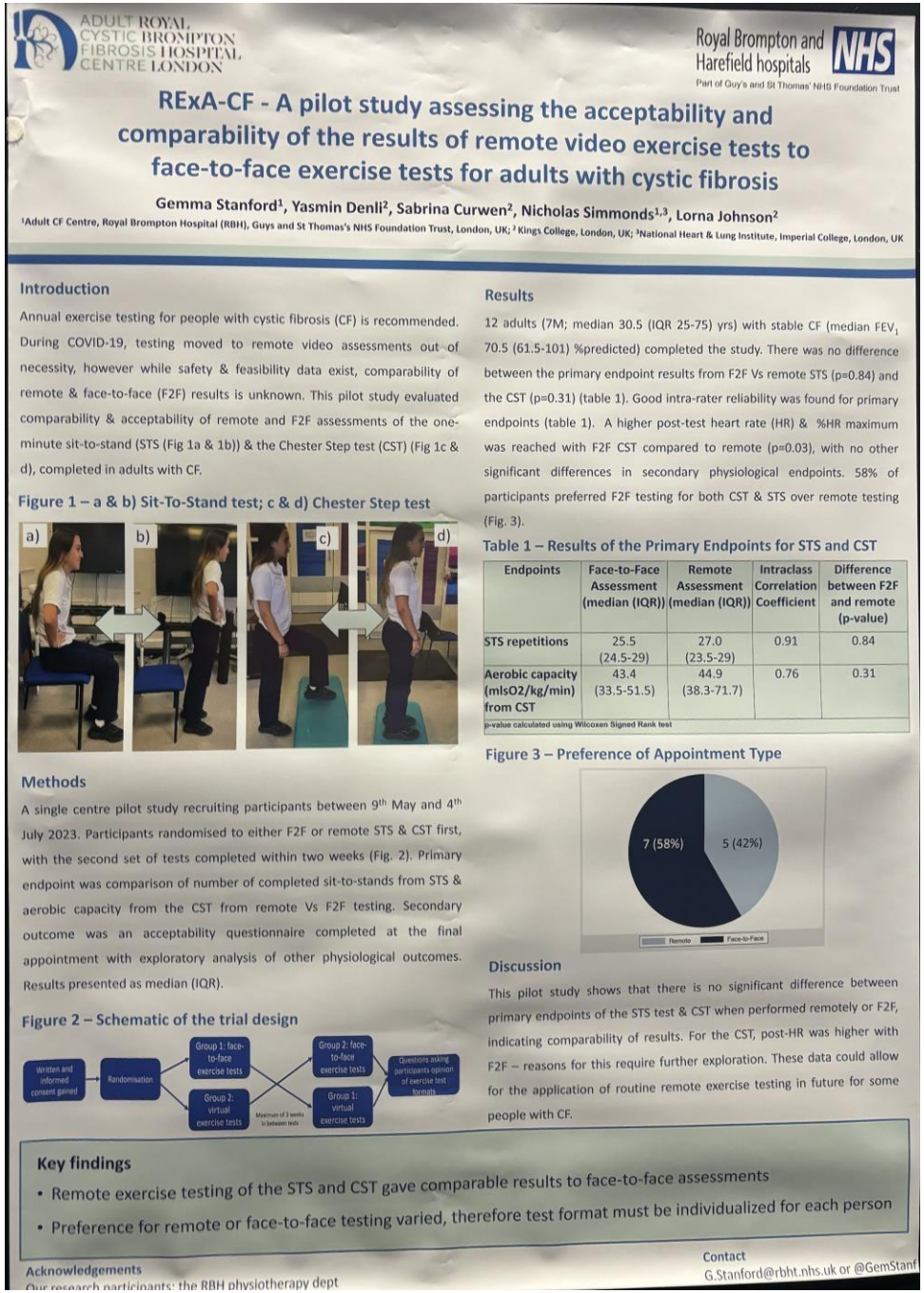
- 66/gp non diff
- Femme 66% Caucasien 91% 34.7 % avec un travail
- 89% ETI en cours
- Sur la dépression 31% modéré à sévère; 41% idées suicidaires
- Diminution significative dépression et anxiété
- Diminution du stress ; vitality = energie pour vivre++ ; activité physique.

CCL: Les patients CF ont 2X plus de signes de dépression avec une mortalité augmentée.

+

EAPA

REXA-CF



ADULT CYSTIC FIBROSIS CENTRE ROYAL BROMPTON HOSPITAL LONDON

Royal Brompton and Harefield hospitals NHS
Part of Guy's and St Thomas' NHS Foundation Trust

RExA-CF - A pilot study assessing the acceptability and comparability of the results of remote video exercise tests to face-to-face exercise tests for adults with cystic fibrosis

Gemma Stanford[1], Yasmin Denli[2], Sabrina Curwen[2], Nicholas Simmonds[1,3], Lorna Johnson[2]

[1]Adult CF Centre, Royal Brompton Hospital (RBH), Guys and St Thomas's NHS Foundation Trust, London, UK; [2] Kings College, London, UK; [3]National Heart & Lung Institute, Imperial College, London, UK

Introduction

Annual exercise testing for people with cystic fibrosis (CF) is recommended. During COVID-19, testing moved to remote video assessments out of necessity, however while safety & feasibility data exist, comparability of remote & face-to-face (F2F) results is unknown. This pilot study evaluated comparability & acceptability of remote and F2F assessments of the one-minute sit-to-stand (STS (Fig 1a & 1b)) & the Chester Step test (CST) (Fig 1c & d), completed in adults with CF.

Figure 1 – a & b) Sit-To-Stand test; c & d) Chester Step test

Methods

A single centre pilot study recruiting participants between 9th May and 4th July 2023. Participants randomised to either F2F or remote STS & CST first, with the second set of tests completed within two weeks (Fig. 2). Primary endpoint was comparison of number of completed sit-to-stands from STS & aerobic capacity from the CST from remote Vs F2F testing. Secondary outcome was an acceptability questionnaire completed at the final appointment with exploratory analysis of other physiological outcomes. Results presented as median (IQR).

Figure 2 – Schematic of the trial design

Results

12 adults (7M; median 30.5 (IQR 25-75) yrs) with stable CF (median FEV_1 70.5 (61.5-101) %predicted) completed the study. There was no difference between the primary endpoint results from F2F Vs remote STS (p=0.84) and the CST (p=0.31) (table 1). Good intra-rater reliability was found for primary endpoints (table 1). A higher post-test heart rate (HR) & %HR maximum was reached with F2F CST compared to remote (p=0.03), with no other significant differences in secondary physiological endpoints. 58% of participants preferred F2F testing for both CST & STS over remote testing (Fig. 3).

Table 1 – Results of the Primary Endpoints for STS and CST

Endpoints	Face-to-Face Assessment (median (IQR))	Remote Assessment (median (IQR))	Intraclass Correlation Coefficient	Difference between F2F and remote (p-value)
STS repetitions	25.5 (24.5-29)	27.0 (23.5-29)	0.91	0.84
Aerobic capacity (mlsO2/kg/min) from CST	43.4 (33.5-51.5)	44.9 (38.3-71.7)	0.76	0.31

p-value calculated using Wilcoxen Signed Rank test

Figure 3 – Preference of Appointment Type

Discussion

This pilot study shows that there is no significant difference between primary endpoints of the STS test & CST when performed remotely or F2F, indicating comparability of results. For the CST, post-HR was higher with F2F – reasons for this require further exploration. These data could allow for the application of routine remote exercise testing in future for some people with CF.

Key findings

- Remote exercise testing of the STS and CST gave comparable results to face-to-face assessments
- Preference for remote or face-to-face testing varied, therefore test format must be individualized for each person

Acknowledgements
Our research participants; the RBH physiotherapy dept

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Les exercices en visio donnent les mêmes résultats qu'en présentiel

Mais la préférence est variable

Impact of 12 months of elexacaftor/tezacaftor/ivacaftor on pulmonary function and exercise capacity in adult patients with cystic fibrosis

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1. Objectives

In December 2022, elexacaftor/tezacaftor/ivacaftor (ETI) was subsidized by the Swedish state for people with cystic fibrosis (CF). Those eligible started ETI in the following months. Currently, the first 12-month follow-ups are being completed.

The aim was to investigate the impact of 12 months of ETI treatment on pulmonary function and exercise capacity in patients with CF.

2. Methods

Data from patients at the Stockholm CF center was collected from the Swedish CF Registry. All patients who performed baseline spirometry and Cardiopulmonary Exercise Test (CPET) before starting ETI and at 12-month follow-up were included. Outcome variables included percent predicted (pp) forced expiratory volume in one second (FEV1), body mass index (BMI), peak oxygen uptake ($ppVO_2peak$), maximum workload (ppWmax), breathing reserve, minute ventilation/carbon dioxide production (VE/VCO_2) slope and capillary partial pressure of carbon dioxide ($PcCO_2$) pre/post CPET.

3. Results

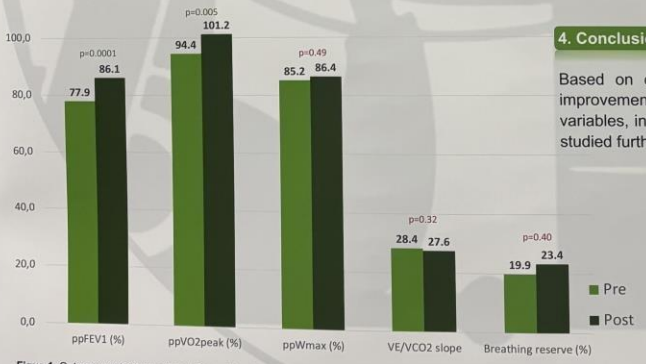
To date, 18 patients (11 male) have completed the 12-month follow-up. Baseline data (mean ± standard deviation) at start: age 27.1 (±7.7) years, BMI 23.4 (±3), ppFEV1 77.9% (±20.5). Ten patients were homozygous for F508del.

Significant improvements were seen in ppFEV1 (+8.2 [±7.1], p=0.0001), $ppVO_2peak$ (+6.8 [±9], p=0.005) and BMI (+1.1 [±1.4], p=0.002). No significant differences were seen in ppWmax (+1.3 [±7.6], p=0.49), breathing reserve (+3.5 [±16.9], p=0.40) or VE/VCO_2 slope (-0.8 [±3.4], p=0.32).

Table 1. Baseline characteristics (n=18)

Age, years (Mean [±SD])	27.1 (±7.7)
Gender, n (Male/female)	11/7
BMI (Mean [±SD])	23.4 (±3)
ppFEV1, % (Mean [±SD])	77.9 (±20.5)
ppFVC, % (Mean [±SD])	91.7 (±16.87)

BMI = body mass index; ppFEV1 = percent predicted forced expiratory volume in one second; ppFVC = percent predicted forced vital capacity



Figue 1. Outcome variables pre/post 12 months of ETI treatment. Data presented as group mean values. ppFEV1 = percent predicted forced expiratory volume in one second; $ppVO_2peak$ = percent predicted peak oxygen uptake; ppWmax = percent predicted maximum workload; VE/VCO_2 = minute ventilation/carbon dioxide elimination

4. Conclusions

Based on our data, 12 months of ETI treatment resulted in improvements in ppFEV1 and $ppVO_2peak$ but other outcome variables, including ppWmax, remained unchanged and should be studied further.

ETI permet d'améliorer en 1 an :

- VEMS
- VO2 max
- Mais restent plusieurs autres paramètres à travailler

Nutrition

OCCURRENCE OF VITAMIN A HYPERVITAMINOSIS IN CHILDREN WITH CYSTIC FIBROSIS AFTER ELEXACAFTOR/TEZACAFTOR/IVACAFTOR THERAPY INITIATION

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INTRODUCTION

Cystic fibrosis-related liver disease and pancreatic dysfunction often lead to fat malabsorption, placing people with CF (pwCF) at risk of fat-soluble vitamin deficiencies. Elexacaftor/tezacaftor/ivacaftor (ETI), a CFTR modulator therapy with notable clinical impact, may influence the absorption of fat-soluble vitamins. Our study aimed to assess fat-soluble vitamin levels pre- and post-initiation of ETI therapy.

METHODS

We analyzed fat-soluble vitamin blood levels, stool elastase measurements, and pancreatic enzyme replacement therapy before and after initiating ETI in pwCF under 18 years. We also examined the characteristics of patients with and without fat-soluble vitamin hypervitaminosis.

RESULTS

1. Involving 29 pwCF initiating ETI therapy (median age 12.0 years) our study revealed a substantial increase in **vitamin D and A levels.**

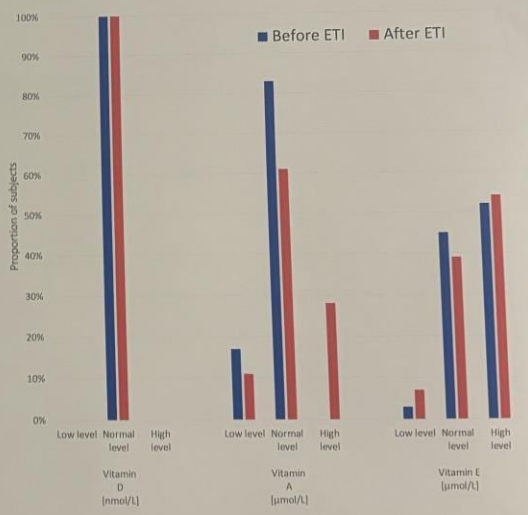
2. Average vitamin D rose from 70.2 (SD 21.2) to 77.8 (SD 25.5) nmol/l (p=0.040), and vitamin A increased from 1.22 (SD 0.34) to 1.57 (SD 0.51) µmol/l (p<0.001).

3. Vitamin E levels remained stable before and after ETI, ranging from 20.9 (SD 7.3) to 22.6 (SD 7.7) µmol/l (p=0.368).

4. While all maintained normal vitamin D levels post-ETI, 28.6% developed vitamin A hypervitaminosis compared to none pre-ETI (p=0.008), particularly in younger patients (median age 10.2 (7.3-12.1) vs. 13.2 (8.7-14.3) years, p=0.038).
No patients reported symptoms of potential vitamin A toxicity.

5. Pancreatic insufficiency persisted with unchanged enzyme dosage post-ETI initiation.

Figure 1. Fat soluble vitamin levels before and after elexacaftor/tezacaftor/ivacaftor initiation



CONCLUSIONS

1. Our study indicates an association between initiating ETI and elevated vitamin D and A levels without affecting pancreatic sufficiency or enzyme replacement therapy.

2. A considerable proportion developed vitamin A hypervitaminosis post-ETI, particularly among younger patients.

3. Vigilant laboratory monitoring is advised for potential vitamin A hypervitaminosis following ETI initiation, particularly as ETI is increasingly prescribed to younger pwCF.

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Attention aux surdosages en vitamine A sous ETI

+

ECBC-bacterio

A comparison of routine cough swabs vs a novel sputum sampling technique in a non-sputum producing CF paediatric population

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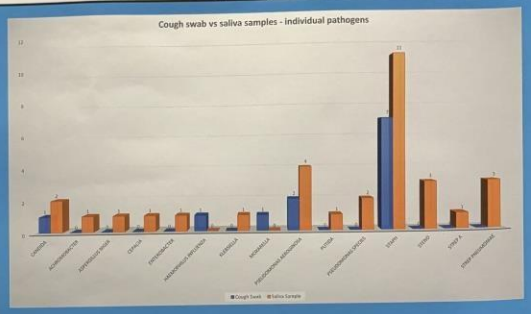
Introduction:

- Routine and exacerbation respiratory cultures are essential in managing children with Cystic Fibrosis (CF).
- Patients on highly effective modulator therapy often struggle to expectorate sputum.
- Current guidelines recommend using cough swabs in non-sputum producing children or induced sputum. (CF standards of care).
- Cough swabs are often not very accurate at capturing lower respiratory pathogens.
- Gold standard recommendation is for induced sputum – lengthy, extra appointment for patient and families, expensive, not always successful in children.

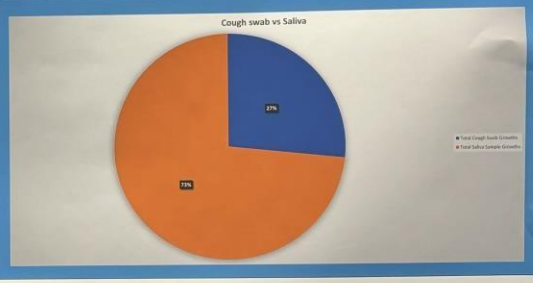


Study Aims:

This study evaluates the efficacy of a novel sputum collection method involving saliva accumulation in the mouth followed by a low to high lung volume huff and cough (Huff/Spit), compared to routine cough swabs.

Methods:

- 70 children, all non-sputum producing, aged 6-18 years, 100 samples over 7-month period.
- Cough swab sample followed by a saliva huff spit sample. Both samples done under supervision of a physiotherapist.
- All samples were cultured according to our enhanced CF protocol.
- All sputum samples exceeded 0.5 mls , four samples rejected for lacking mucoid or purulent content.



Results:

- 17 Huff spit samples yielded CF pathogens that weren't seen on the cough swabs.
- 3/17 symptomatic patients – changed Abx therapy because of CF pathogen found on Huff spit sample.
- 14/17 Asymptomatic patients – 10 started Abx therapy due to CF pathogen found on Huff spit sample.
- Pseudomonas aeruginosa was identified in 4 non-symptomatic Huff spit samples and only 2 corresponding cough swab samples.
- Chi-square analysis showed a significant difference in bacterial detection between the new method and cough swab tests ($\chi^2 = 5.03$, $p = 0.025$).

Conclusion:

- Our technique for obtaining lower respiratory samples for culture was easy to teach and quick to perform in this age group.
- Results show superior detection rates with the huff spit tests compared to cough swabs.
- This approach has the potential to significantly improve respiratory sample collection for CYP with CF, minimizing patient burden while enhancing diagnostic accuracy.
- Further research looking at how the Huff spit test compares to induced sputum and then bronchoscopy would be beneficial.

References:

- Ronchetti K, Tame JD, Paisey C, Thia LP, Doull I, Howe R, Mahenthiralingam E, Forton JT. The CF-Sputum Induction Trial (CF-SpIT) to assess lower airway bacterial sampling in young children with cystic fibrosis: a prospective internally controlled interventional trial. Lancet Respir Med. 2018 Jun;6(6):461-471. doi: 10.1016/S2213-2600(18)30171-1. Epub 2018 May 16. PMID: 29778403; PMCID: PMC5971213.
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- FORTON, J.T., 2019. Detecting respiratory infection in children with cystic fibrosis: Cough swab, sputum induction or bronchoalveolar lavage. Paediatric Respiratory Reviews, 31, pp. 28-31.

Comparaison écouvillon vs toux-salive :

Facile pour les enfants
Meilleure détection des pathogènes comparés aux écouvillons

A Service Improvement Project Comparing Bacterial Growth in Standard Routine Cough Swabs at Annual Review Compared to an Induced Sputum in Children with Cystic Fibrosis

Sheffield Children's NHS Foundation Trust

Aims

To evaluate whether performing an induced sputum (I.S.) as part of our annual review process is a more effective way of sampling our patients than doing a routine cough swab. The aim was to compare the bacterial growth on a routine cough swab to that of an induced sputum performed on the same day.

Background

Since the advancements of CFTR modulators, clinicians have been finding that people with CF are generally less productive of sputum. This has made sampling patients for bacterial growth harder.

The CF SPiT study (Ronchetti, 2018) states,"sputum induction is superior to cough swab for pathogen detection and is effective for sampling the lower airway and is a credible surrogate for BAL in symptomatic children". Because of this study and due to the reduced amount of sputum samples we found we were able to obtain, we decided to develop this service evaluation of our sampling methods at annual review.

The previous method of sampling at Sheffield Children's Hospital (SCH) was to obtain a cough swab for microbiology, cultures and sensitivities (M,C&S) at every clinic review, obtain a cough swab for M,C&S and acid fast bacilli (AFB's) at every annual review and to do an induced sputum when clinically indicated.

We found a general decrease in the quantity of growths of bacteria following the introduction of Kaftrio. This led us to question if the bacteria e.g. pseudomonas aeruginosa is no longer present, or are we just not detecting it?

This provided us with the hypothesis for this service evaluation.

We decided to compare the bacterial growth on a routine cough swab taken at annual review to that of an induced sputum performed on the same day. Depending on the results of the project, there was potential to develop the service to include an induced sputum as part of every patient's annual review process.

Method

We included every patient over the age of 6 years old. A routine cough swab was obtained and sent for M,C&S and AFB's. An induced sputum was then performed using 4mls of 7% hypertonic saline via either the patients standard compressor/Eflow or Ineb or via the oxygen port on the wall. If a patient sounded like they may produce something at the end of the 4mls we would give them another 4mls of hypertonic saline. During the induced sputum we would encourage breathing techniques, use of adjuncts and some exercise e.g. star jumps or bouncing on a trampette to encourage expectoration. Any sputum obtained was sent for M,C&S and AFB's along with the cough swab.

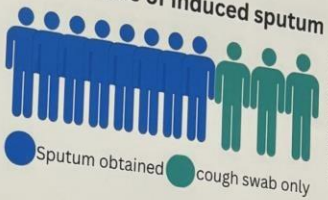
Results

So far we have perfomed 26 Induced sputums at annual review, 19 of these were successful and sputum was obtained.

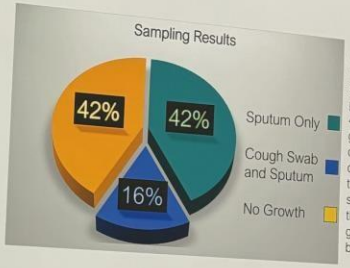
Bacteria detected included MRSA, pseudomonas aeruginosa, staphylococcus aureus, achromobacter and haemophilus influenza. These are all bacteria that would we offer a course of oral antibiotics for. It is also worth noting that 3 of the samples that grew on the cough swab and the sputum, grew a heavy growth on the sputum and only a light growth on the cough swab.

The sampling results for the induced sputum's and cough swabs obtained are represented in the following graphs:

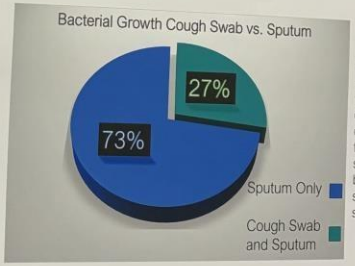
Success rate of induced sputum



Out of the 26 Induced sputum's performed, 73% yielded a sputum sample that was sent for bacterial testing



Out of the 26 Induced sputum's performed, 42% of them grew bacteria on the sputum's only but not on the cough swabs, 42% of the samples grew no bacteria at all.



73% of the 11 positive samples were detected on the sputum only, only 27% of the positive samples grew on both the cough swab and the sputum.

Conclusion

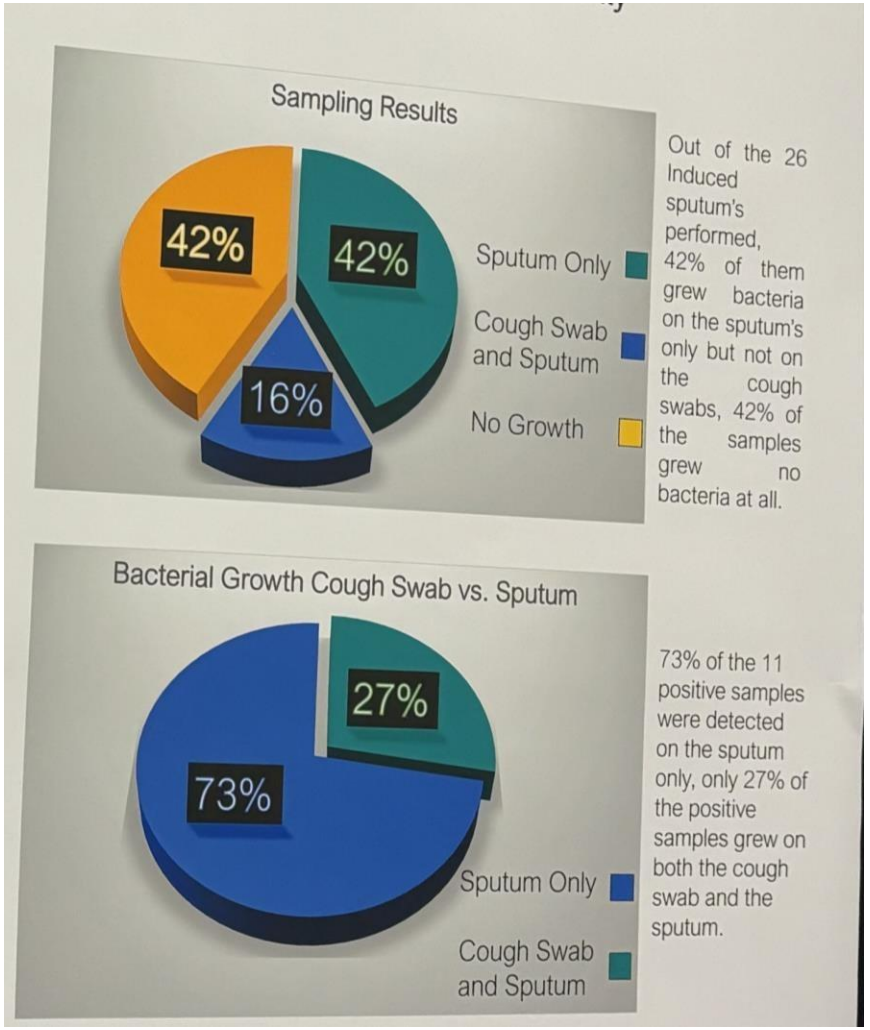
The results of this service improvement project support our hypothesis that Induced sputum's can detect bacteria more effectively than a cough swab.

Adding Induced sputum's into the annual review process is a relatively quick and cost effective way of improving respiratory sampling at Sheffield Children's Hospital. Induced sputum has been well tolerated in children over the age of 6 and is often successful in obtaining a sample, even in well patients with no current respiratory symptoms.

Reference

Ronchetti, K., Tame, J., Paisey, C., Thia, L. P., Doull, I., Howe, R., Mahenthiralingam, E., & Forton, J. T. (2018). The CF-Spectum Induction Trial (CF-SpIT) to assess lower airway bacterial sampling in young children with cystic fibrosis: a prospective internally controlled intervention trial. *Lancet Respiratory Medicine, 6(6).* 461-471.

Les écouvillons passent à côté des colonisations par rapport aux ECBC induits



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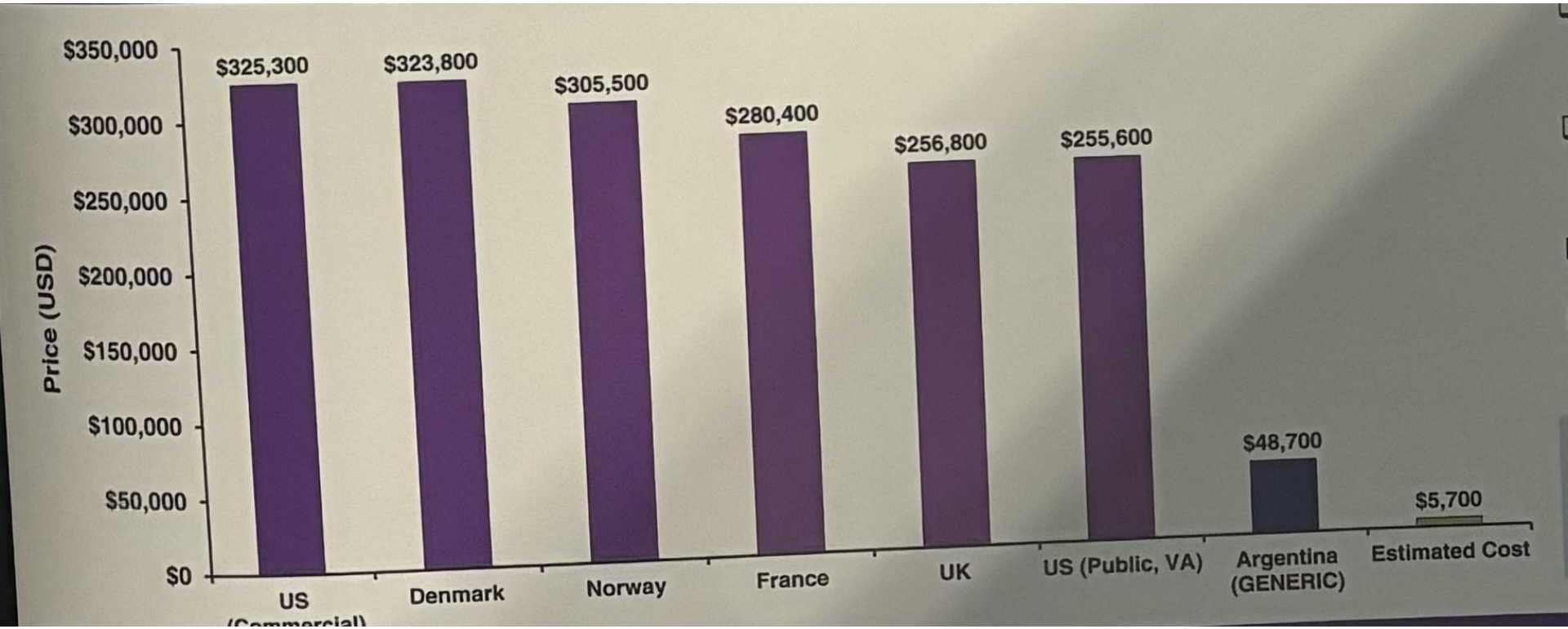
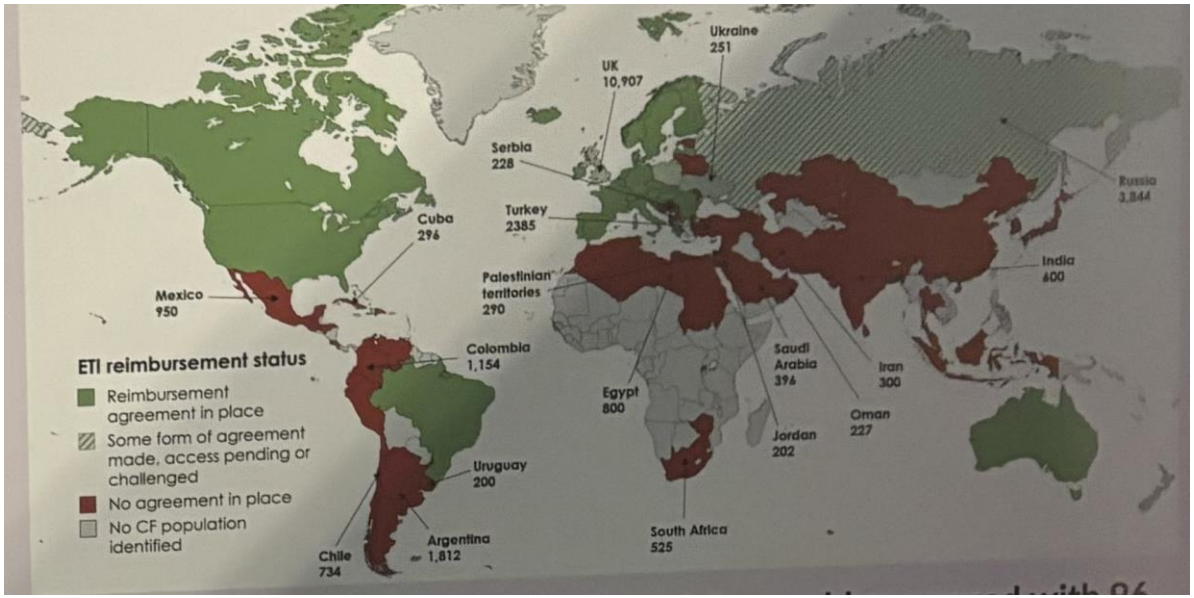
coût

Cout variable dans le monde



Accès complexe à l'ETI pour beaucoup de pays



Experience de l'Afrique du Sud qui associe l'ETI à la Clarythromycine

- 250mg/j diminution de moitié
- 500mg/j Deux prises par semaine