ADHERENCE:

A Key to SUCCESSFUL TREATMENT of Cystic Fibrosis

Final Outcomes Report
March 17, 2023 – March 17, 2024
Grant ID: FR-009532

Supported by an educational grant from Genentech, a member of the Roche Group

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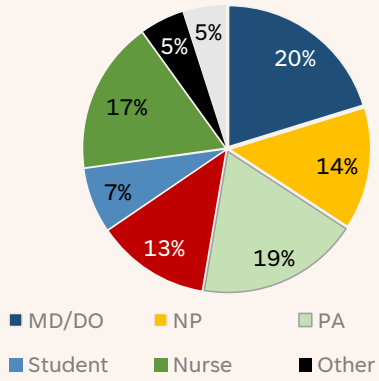
ADHERENCE: A KEY TO SUCCESSFUL TREATMENT OF CYSTIC FIBROSIS

Enduring activity on Epocrates
March 17, 2023 – March 17, 2024
https://online.epocrates.com/contextuallink/cme/activity/KZTKFE

- **Panel Discussion to Address Identified Provider Needs**
- **Patient Testimonial**
- **Provider Resources**

PARTICIPATION



MD/DO = 277
NP = 190
PA = 252
PharmD/Tech = 175
Student = 100
Nurse = 235
Other HCP = 63
Other = 60
Total Test-takers = 1,352

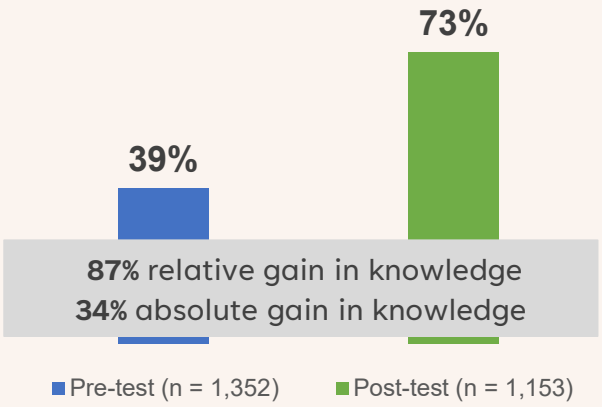
Learner Guarantee	Learner Actual
1,300	2,024

Potential Impact To 435,880* Patient Touchpoints This Year

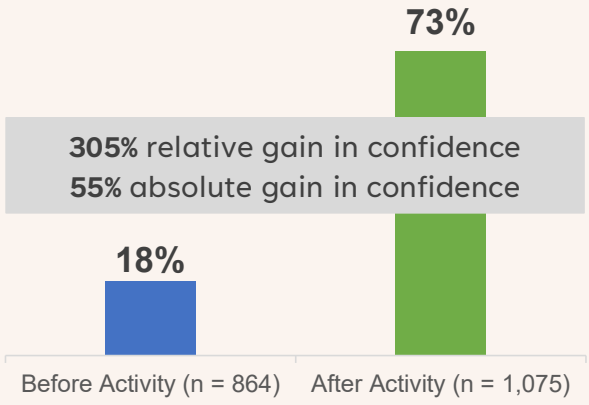
**Based on 403 evaluation respondents who reported number of weekly patient visits for CF. This estimate is for visits, not individual patients seen.*

KNOWLEDGE AND CONFIDENCE IMPROVEMENTS

Overall Knowledge Gain

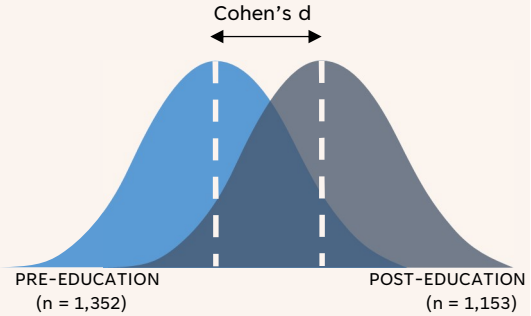


Overall Confidence Gain



OVERALL EFFECT

Overall, this activity led to an effect size of 1.27, indicating a knowledge/competence shift of **64%** compared from pre-activity to post.



INTERPRETATION OF COHEN'S D EFFECT SIZE

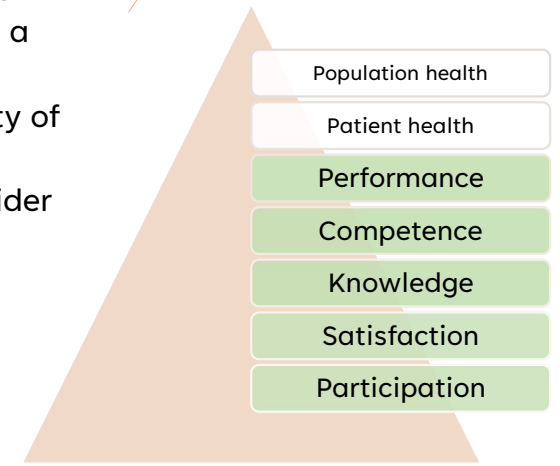
Assessment researchers often use general guidelines to help interpret Cohen's d – small (0.2), medium (0.5), and large (0.8). For more information on interpreting Cohen's d, refer to https://rpsychologist.com/cohend/

76%
n = 1,075

Respondents who intend to make changes to practice as a result of the activity

ACTIVITY OVERVIEW AND OUTCOMES METHODOLOGY

The approval of CFTR modulators has revolutionized the treatment of people with CF. However, their use alongside conventional therapies such as antibiotics, mucolytics, and bronchodilators remains a point of confusion among many healthcare providers. In this activity, expert faculty review CF pathophysiology and therapies targeting infections and lung function and improving patient quality of life. Faculty also address concerns with drug complexity, burden, and adherence. National Jewish Health collaborated with the Cystic Fibrosis Foundation to develop and distribute a survey of provider and patient needs with regard to CF treatment strategies and adherence. Results of the survey informed content development and were addressed in an engaging panel discussion with faculty.



Target Audience: Adult and pediatric pulmonologists, primary care physicians, APPs, pharmacists and nurses who manage patients with cystic fibrosis.

Moore's Levels 1 (Participation) and 2 (Satisfaction): course evaluation
Levels 3, 4 (Knowledge – Competence): pre-post survey
Level 5 (Performance): a 30-day follow-up survey of the learners compared to a non-learner control

ACCME ACCREDITED WITH COMMENDATION

National Jewish Health designates this enduring material for a maximum of 1.75 *AMA PRA Category 1 Credits*™.

Provider approved by the California Board of Registered Nursing, Provider Number 12724 for 1.75 contact hours

ACPE

National Jewish Health designates this enduring material for 1.75 contact hours (0.175 CEUs) of the Accreditation Council for Pharmacy Education.

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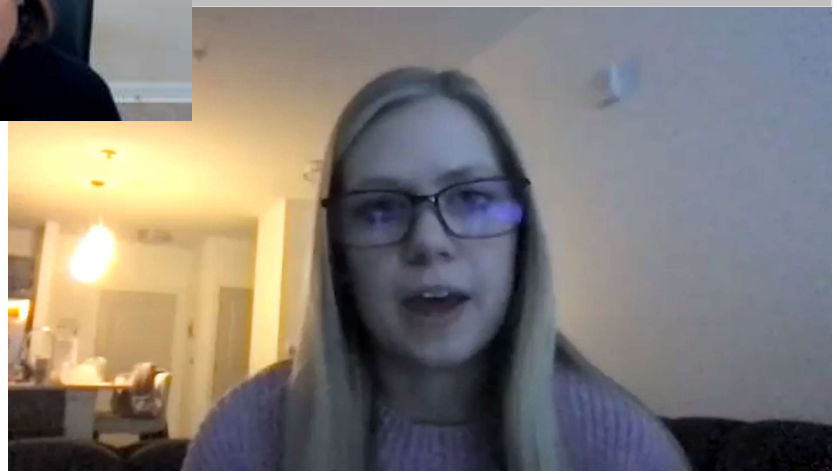
"All of the presenters were excellent, providing real-world information and experience."
-Online participant

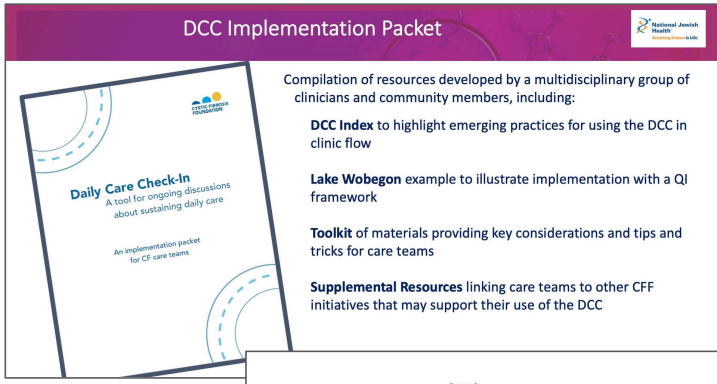
PROGRAM FEATURES

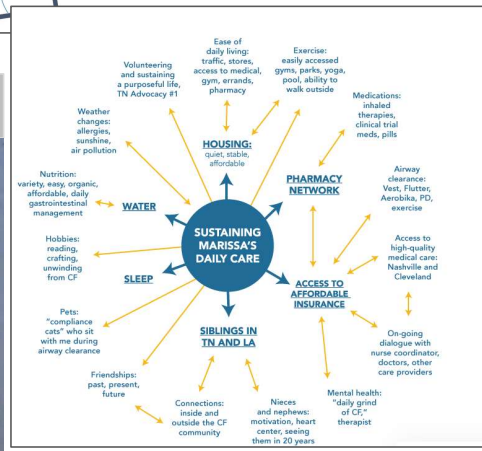
Panel Discussion to Address Identified Provider Needs

Patient Testimonial



Provider Resources





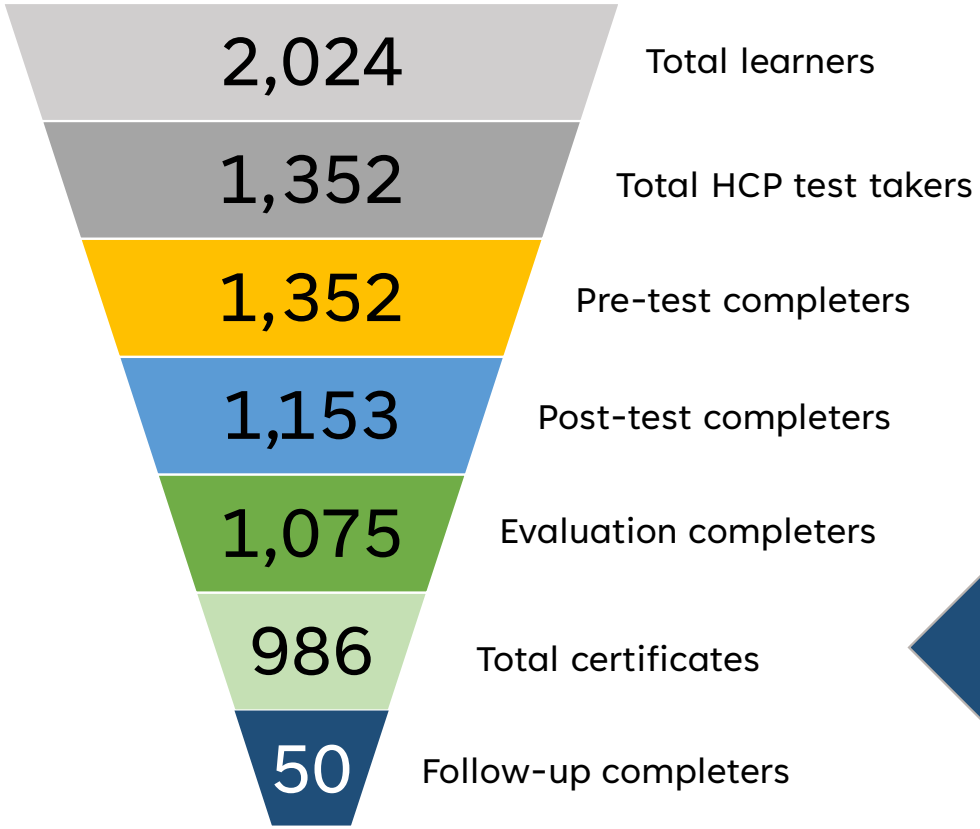
LEARNING OBJECTIVES

1. Evaluate the role of CF therapies in clinical management of lung infections, pulmonary exacerbations, and preservation of lung function

2. Analyze new and emerging clinical trial and real-world data on pulmonary outcomes for CF therapies

3. Utilize evidence-based strategies to improve treatment adherence in CF

PARTICIPATION FUNNEL

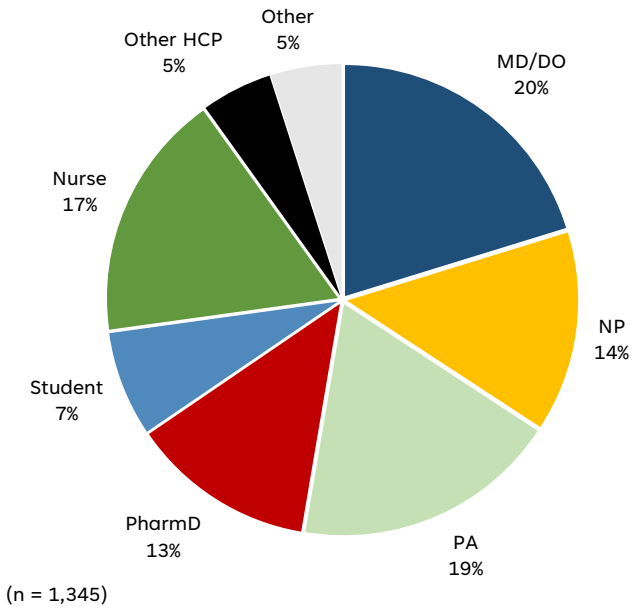


Exceeded learner guarantee by **724 learners!**

Exceeded certificate guarantee by **586 certificates!**

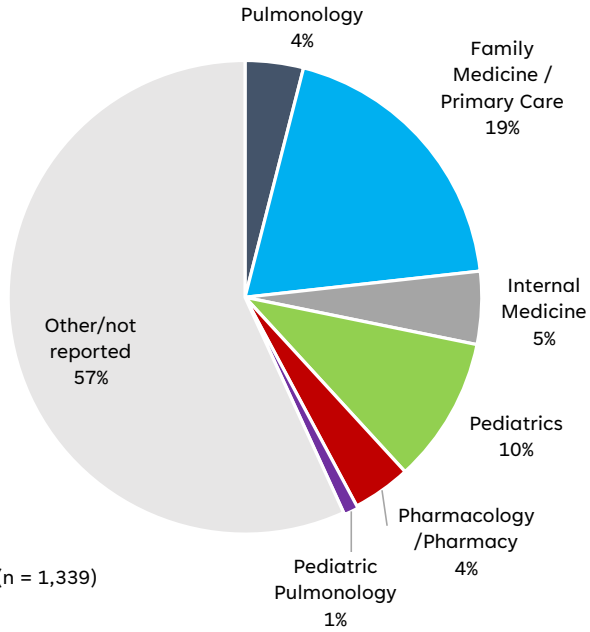
PARTICIPATION – TOTAL HCP TEST TAKERS (N = 1,352)

Profession



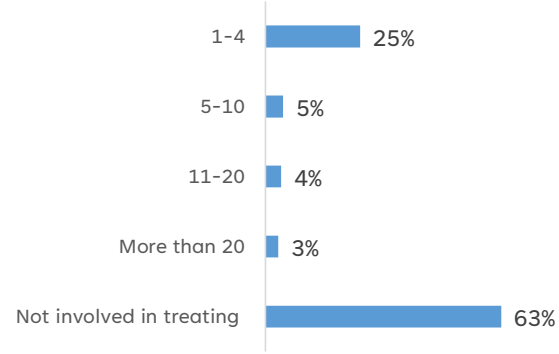
(n = 1,345)

Specialty



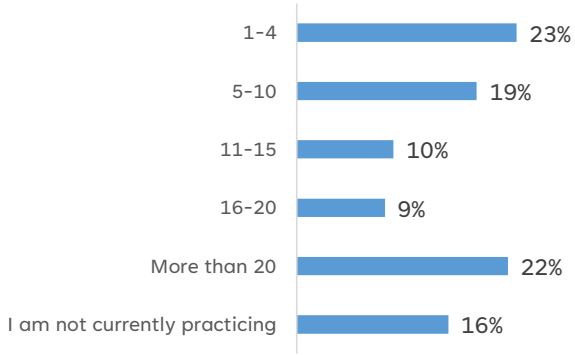
(n = 1,339)

Patients with CF treated per week



(n = 1,075)

Years in Practice



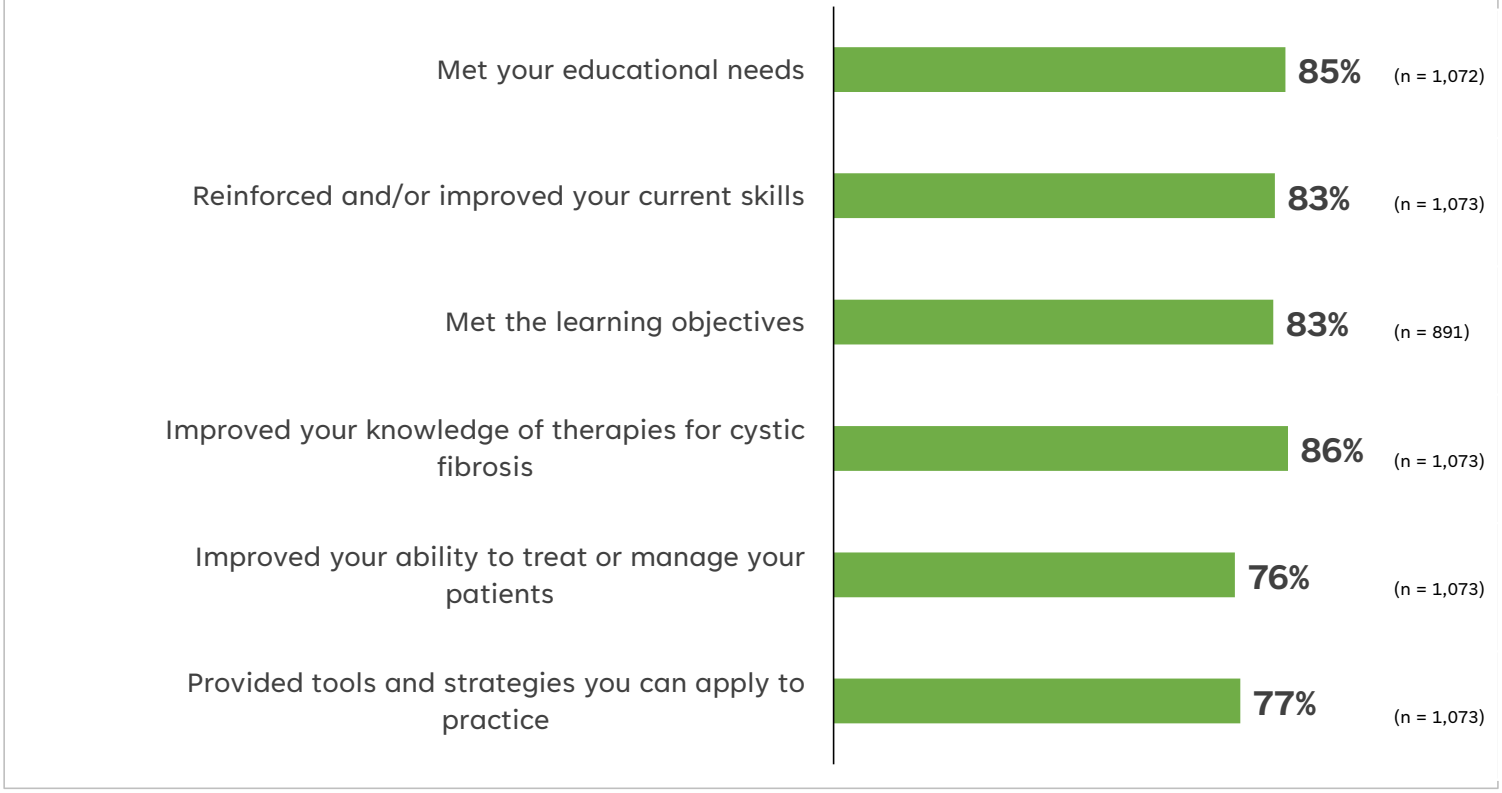
(n = 1,075)

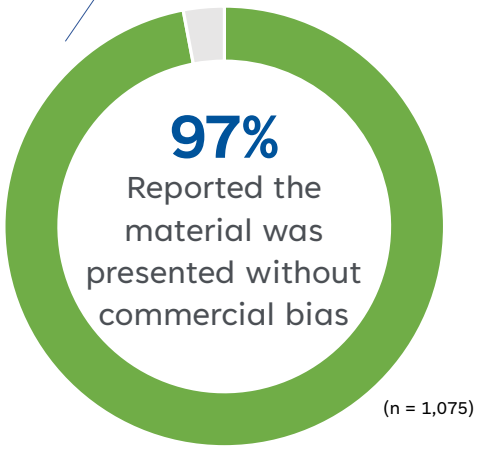
Despite targeted marketing efforts, this activity had a lower percentage of learners in the target audience than anticipated. However, the program faculty reported that this education is highly beneficial for healthcare providers across many specialties:

> *"Because patients with CF are living longer, we need more healthcare providers across specialties to have an understanding of CF management, as they are seeing CF patients for care in other areas."*
> – Jennifer Taylor-Cousar, MD, MSCS, ATSF Activity Program Chair

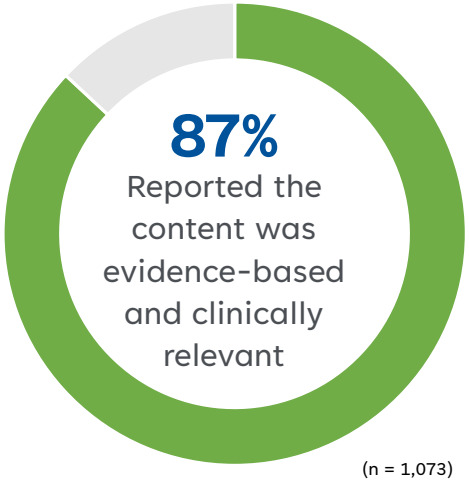
EVALUATION – SATISFACTION

Evaluation respondents agreed with the following statements about the activity: (agree, strongly agree)





(n = 1,075)



(n = 1,073)

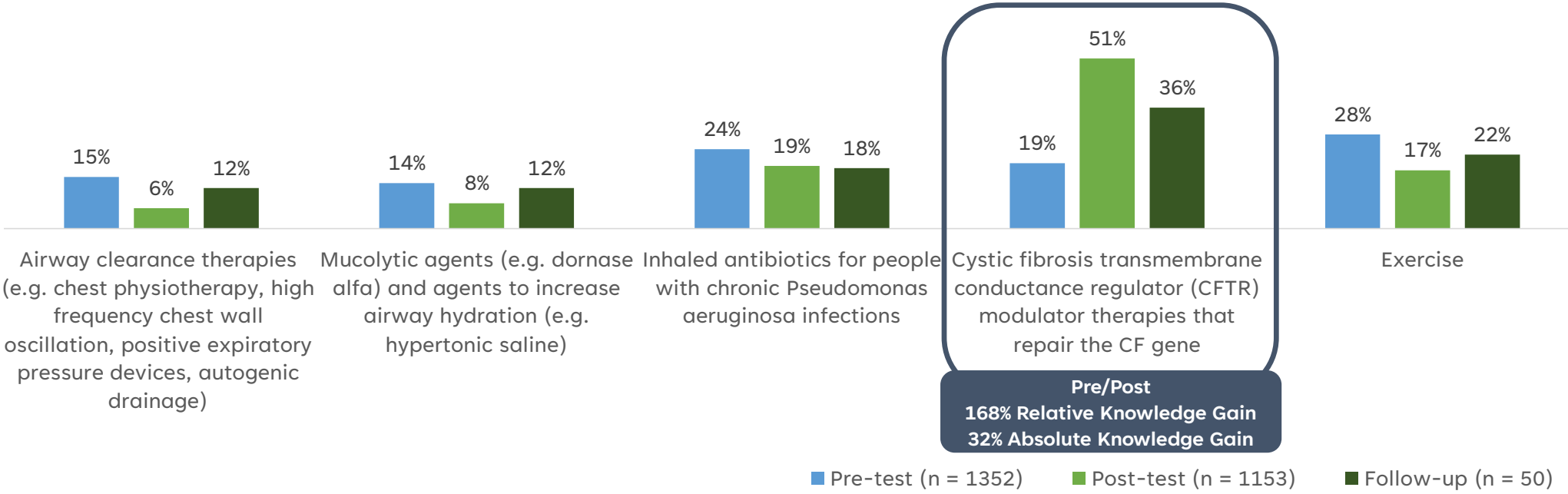
What prompted you to participate in this CME activity?

(open-ended question, direct quotes)

- “2 grandkids with CF”
- “Interested in the community treatment of CF”
- “received email and like to stay up to date”
- “Keep up with new developments”
- “Lack of sufficient knowledge in taking care of CF patients”
- “Intellectual curiosity and had first CF patient in a long time”
- “Wanted update on CF care”
- “Have worked with CF patients off and on for years starting when I was an MA in pediatrics 30 years ago”
- “I see a handful of CF related diabetes cases on my inpatient diabetes consult service and would like to be more familiar with their diagnosis.”
- “Continued improvements / changes in management”
- “Interested in learning more about Patient management and plan of care with those who have CF”
- “Credit and to learn more about something I don’t Tx or deal with in clinic since all are referred out”
- “Learn about new treatment options for CF”
- “I would like to gain more knowledge about CF as I typically do not see them in my office.”
- “Learn more on a subject I didn’t know much about”
- “To get an idea of treatment choice”

OUTCOMES ASSESSMENT – KNOWLEDGE

Current recommendations for the treatment of chronic lung disease in people with cystic fibrosis (CF) include all of the following therapies except:



Learning Objectives Addressed:

1

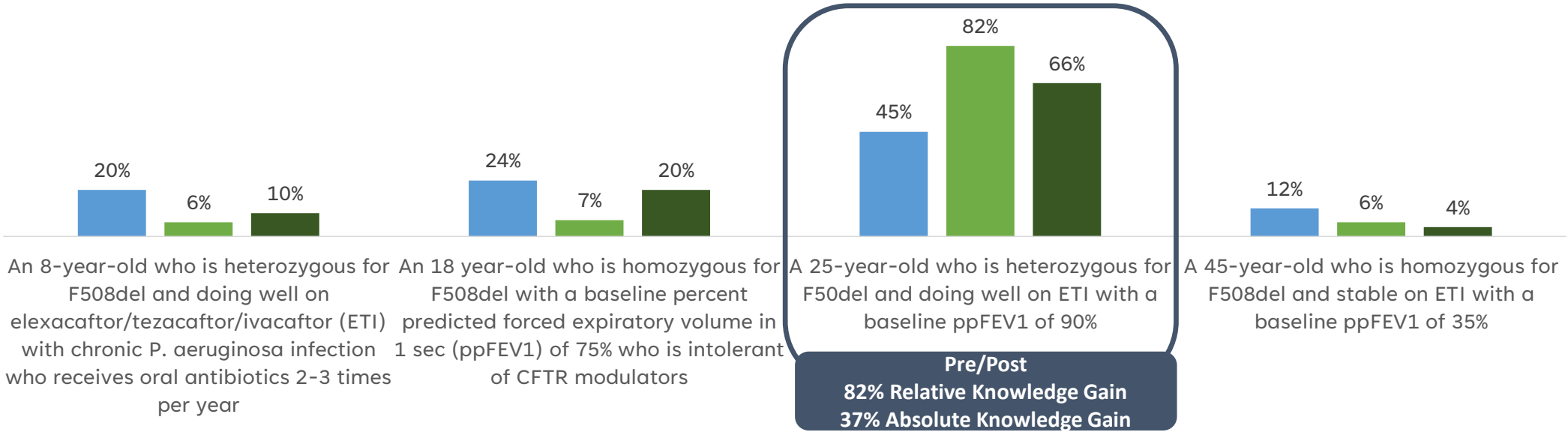
Exercise, airway clearance therapies, mucolytic agents, agents to increase airway hydration, and inhaled antibiotics are recommended for people with chronic CF lung disease. While CFTR modulator therapies are available for those with qualifying CFTR variants, CFTR modulators act on the CFTR protein, not on the CFTR gene.

PRE vs POST $P < .001$

PRE vs FOLLOW-UP $P = .004$

OUTCOMES ASSESSMENT – KNOWLEDGE

Based on the results of discontinuation versus continuation of hypertonic saline or dornase alfa in modulator-treated people with CF in the SIMPLIFY study, which of the following individuals with CF would be the most appropriate to consider discontinuation of hypertonic saline or dornase alfa?



Learning Objectives Addressed:

2

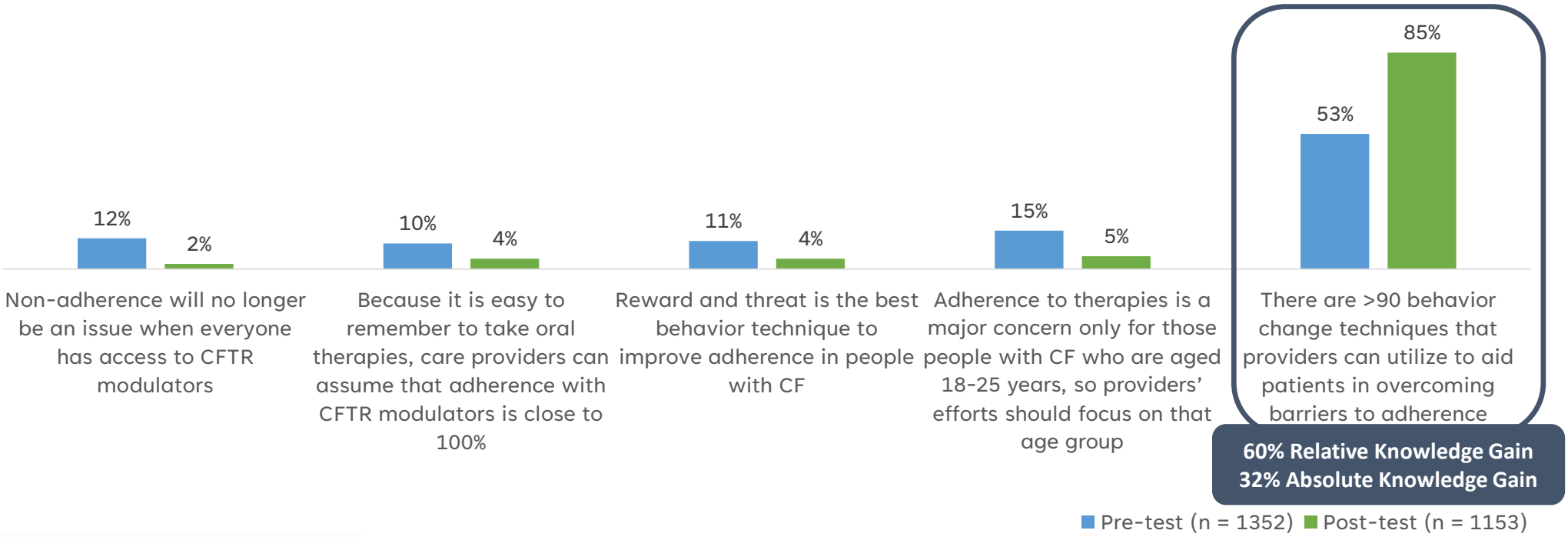
Key inclusion criteria in the SIMPLIFY study included people who were ≥12 years of age, had mild-moderate CF lung disease and were actively being treated with ETI. Only the individual described in the highlighted box meets those criteria.

PRE vs POST *P* < .001

PRE vs FOLLOW-UP *P* = .003

OUTCOMES ASSESSMENT – KNOWLEDGE

Which of the following is true when considering evidence-based strategies to improve treatment adherence?



Learning Objectives Addressed:

3

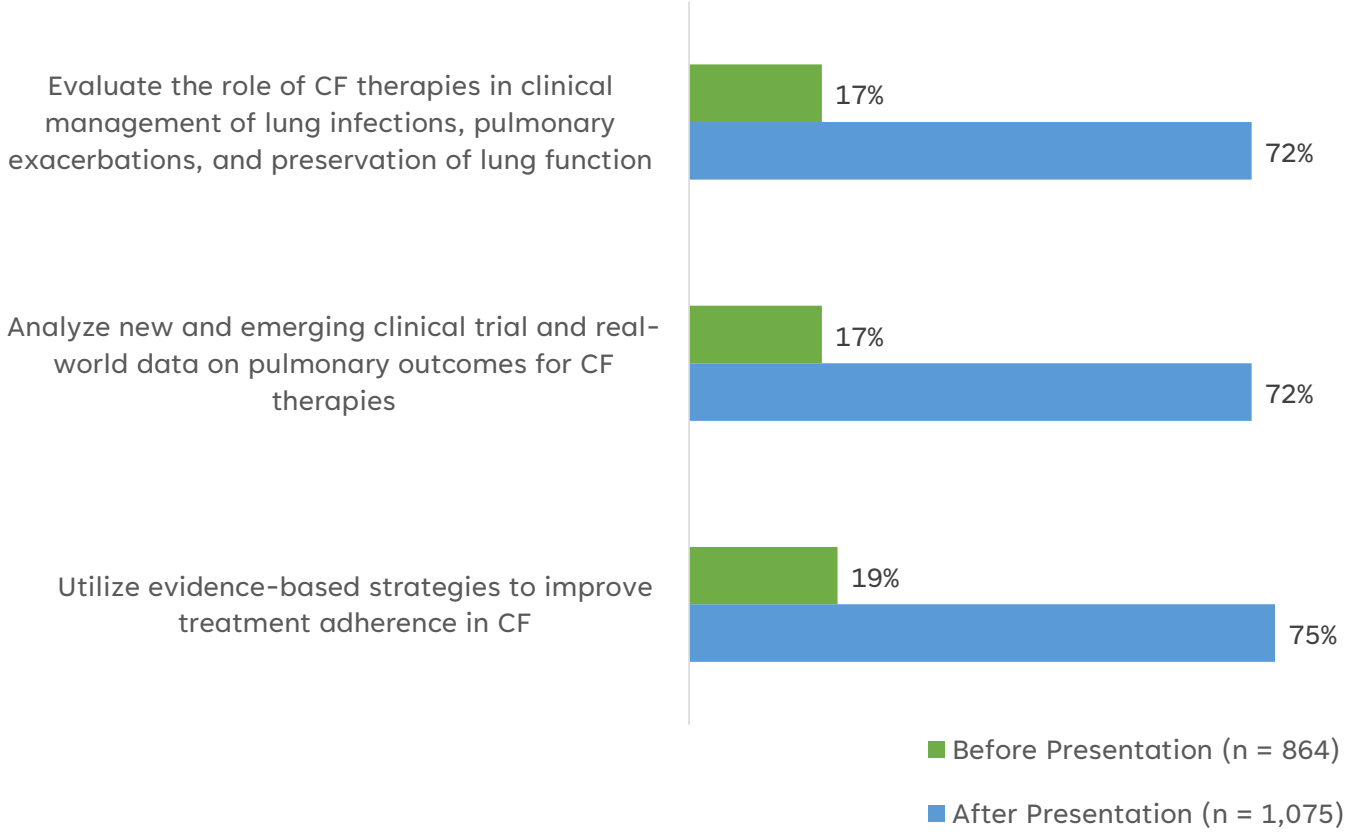
Adherence is an issue for people of all ages and remains an issue even for many of those who are taking CFTR modulators. It is important to identify the barriers for an individual's adherence in order to decide which behavior change technique is most likely to be effective for that individual. More than 90 techniques (with 16 categories) have been described to impact behavior change.

PRE vs POST $P < .001$

Note: this question was not included in the follow-up survey.

OUTCOMES ASSESSMENT – CONFIDENCE

Evaluation respondents reported their confidence as it relates to the learning objectives before and after the activity (% somewhat - very confident)



> *"The personal example of the young woman who has lived with CF her entire life and the ups and downs and success story would be relatable to tell my patients."*
>
> - Online participant

PRE vs POST $P < .001$

OUTCOMES ASSESSMENT – CHANGES IN PRACTICE

What changes will you make in practice as a result of what you learned in this activity?

Evaluation and monitoring
- FEV evaluations
- Appropriate work-up and initial treatment
- Monitoring extent of pulmonary infections to assess level of treatment required

Treatment of patients with CF
- Utilize evidence-based guidelines for managing treatment
- More aware of all the possible treatment options for CF patients
- Evaluate summaries for studies like PROMISE and BEGIN
- Better able to determine available treatment options
- Recommend exercise in CF patients
- Encourage modulator therapy
- Stay up to date with new CFTR modulator therapies

Adherence to treatment regimens
- Analysis of barriers to treatment compliance
- Show more empathy for non-adherence issues
- Improved patient consultation and support
- Reinforcing of treatment information and encouragement of treatment regimen

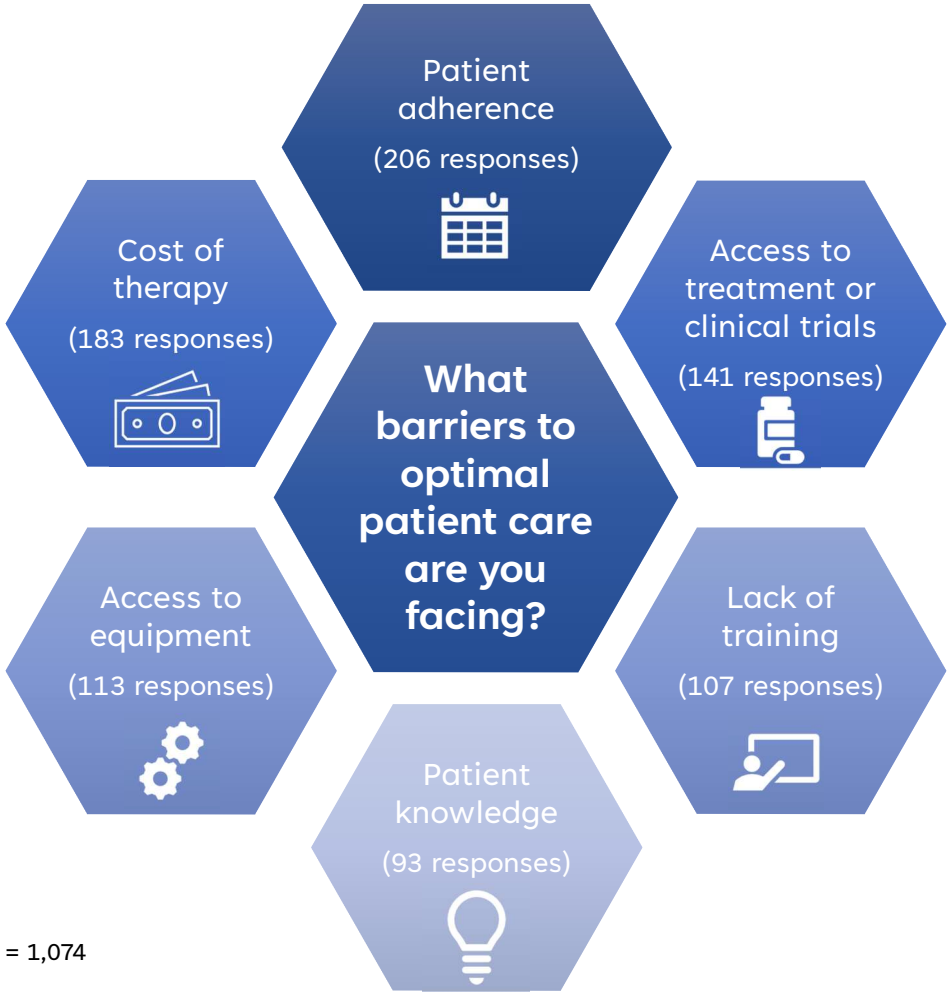
Referral
- Know when to refer
- Working closely with pulmonologists

76%

n = 1,075

of evaluation respondents intend to make changes in practice as a result of the activity

OUTCOMES ASSESSMENT – BARRIERS



n = 1,074

79%

n = 918

of evaluation respondents report the activity will help to address barriers to optimal patient care

81%

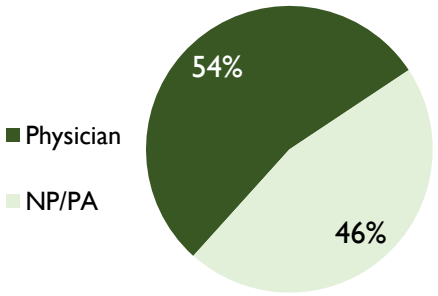
n = 1,075

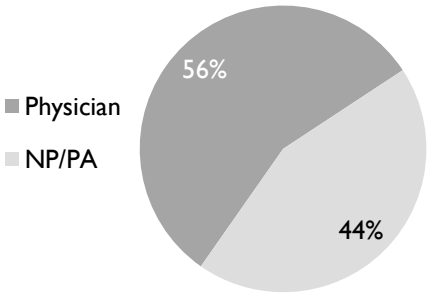
of evaluation respondents report they are likely to use the patient education resources in practice

FOLLOW-UP/CONTROL – DEMOGRAPHICS (N = 50)

A sample **of 50 MD/DO/NP/PA learners** and a demographically similar control group of **non-learners (n = 50)** were collected for this assessment.

Degree



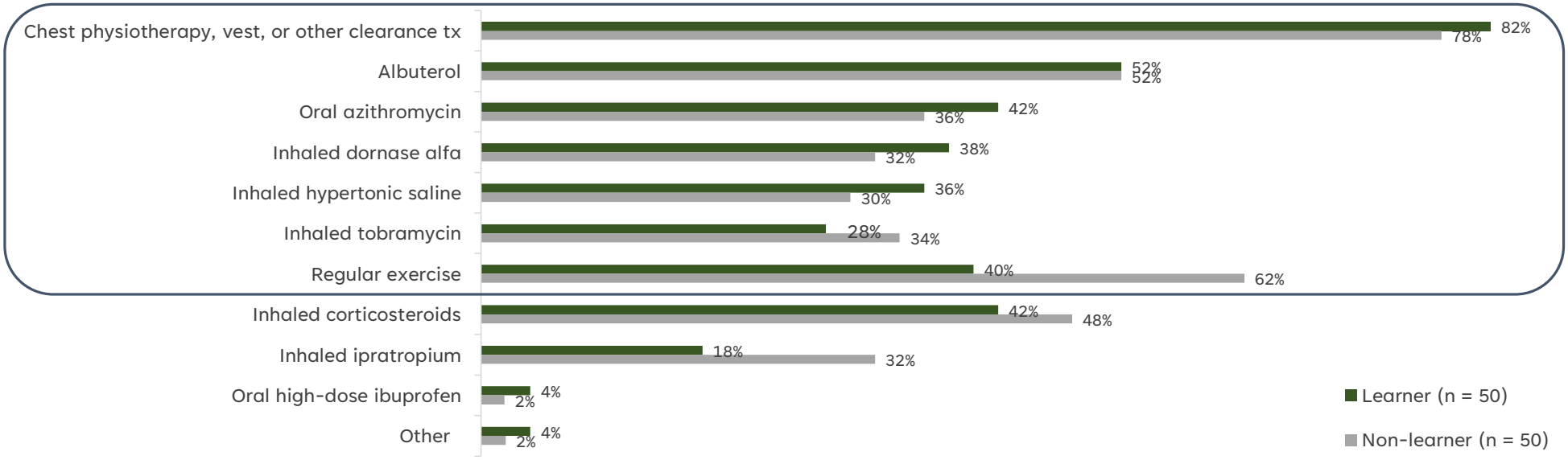


	Learner (n = 50)	Non-learner (n = 50)
Specialty		
Pulmonology	14%	12%
Pediatric pulm	2%	2%
Primary care	48%	74%
Pediatrics	16%	12%
Other*	20%	0%
Number patients seen/week, mean	71	78
Number of CF patients seen in past 6 months	4	4
Years in practice, mean	19	21
Practice location		
Urban	36%	44%
Suburban	44%	46%
Rural	20%	10%
Present employment		
Solo practice	22%	22%
Group single-specialty practice	32%	38%
Group multi-specialty practice	20%	22%
Academic/university/medical school	8%	4%
Non-government community hospital	14%	8%
Government/military/VA hospital	2%	0%
Other**	2%	6%

PERFORMANCE ASSESSMENT – RECOMMENDED THERAPIES

Case #1: An 18-year-old woman with cystic fibrosis (CF) presents for a routine follow-up visit. She was diagnosed with CF at 2 months of age based on the results of her newborn screen/sweat testing. Her most recent FEV1 is 55% predicted. She has pancreatic insufficiency and has had difficulty gaining weight in the past. Her current BMI is 18 kg/m^2. She is chronically infected with *Pseudomonas aeruginosa* and has exacerbations of her CF 3 to 4 times per year requiring oral antibiotics and increased airway treatment at home. She has been hospitalized 3 times (ages 5, 11, and 14 years old) for IV antibiotics. She is homozygous for the F508del variant and takes elexacaftor-tezacaftor-ivacaftor (ELX-TEZ-IVA), as well as pancreatic enzymes, fat soluble vitamin supplementation, and adheres to a high fat, high protein diet.

Which therapies for lung disease would you recommend for this patient? (select all that apply)



Learning Objectives Addressed:

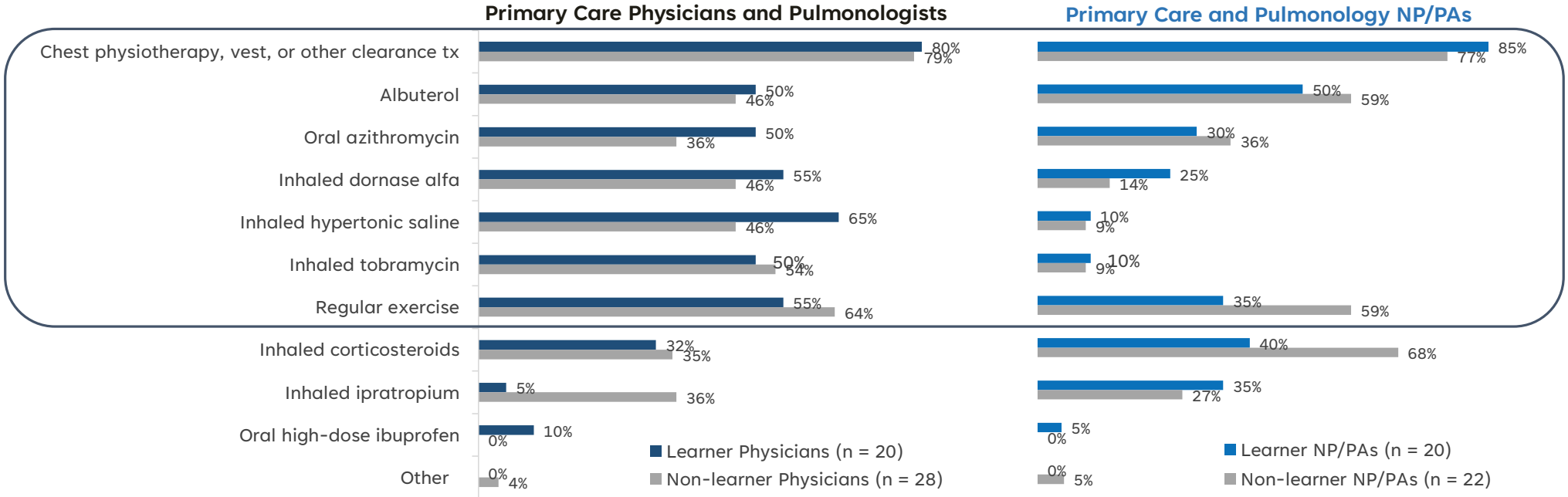
1

All of these are appropriate options for this patient except inhaled corticosteroids, ipratropium, and ibuprofen.
Fewer learners indicated use of inhaled corticosteroids or ipratropium.
Most clinicians are recommending a physical clearance treatment, with no more than half choosing a specific pharmacologic treatment.

PERFORMANCE ASSESSMENT – TREATMENT APPROACH BY ROLE

Case #1: An 18-year-old woman with cystic fibrosis (CF) presents for a routine follow-up visit. She was diagnosed with CF at 2 months of age based on the results of her newborn screen/sweat testing. Her most recent FEV1 is 55% predicted. She has pancreatic insufficiency and has had difficulty gaining weight in the past. Her current BMI is 18 kg/m^2. She is chronically infected with *Pseudomonas aeruginosa* and has exacerbations of her CF 3 to 4 times per year requiring oral antibiotics and increased airway treatment at home. She has been hospitalized 3 times (ages 5, 11, and 14 years old) for IV antibiotics. She is homozygous for the F508del variant and takes elexacaftor-tezacaftor-ivacaftor (ELX-TEZ-IVA), as well as pancreatic enzymes, fat soluble vitamin supplementation, and adheres to a high fat, high protein diet.

Which therapies for lung disease would you recommend for this patient? (select all that apply)



Learning Objectives Addressed:

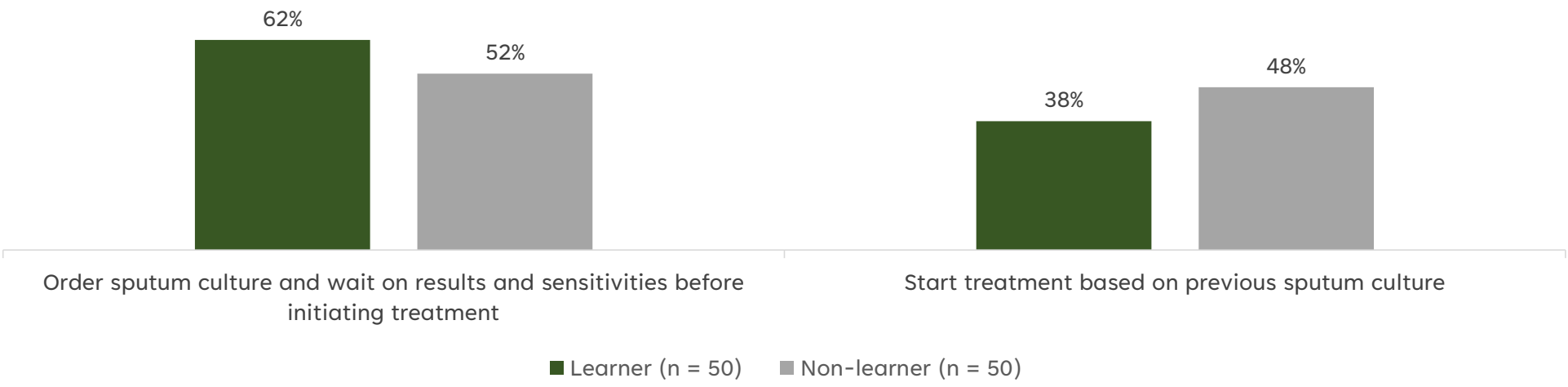
1

While gains were seen amongst learners, clinicians, and NP/PAs in particular, could benefit from additional future education addressing appropriate pharmacologic options and the benefits of regular exercise. Corticosteroids may be overused in this patient population, particularly by NP/PAs.

PERFORMANCE ASSESSMENT – CULTURE OR TREAT?

Case #1 continued: Two months later, the patient had an exacerbation and was treated with ciprofloxacin for two weeks. You see the patient in your office two weeks after this, and she presents with increasing cough, fatigue, and worsening shortness of breath. She reports good adherence with her medication regimen, which consists of ELX-TEZ-IVA, twice daily inhaled albuterol, dornase alfa, hypertonic saline, and tobramycin (every other month), twice daily chest physiotherapy, and oral azithromycin three times weekly. Her FEV1 at this visit is 50% predicted. Her most recent sputum culture from one month ago showed *Pseudomonas aeruginosa*.

Which of the following would you do next?



Learning Objectives Addressed:

1

While there is no evidence-based choice in this decision, learners are more likely to wait on the results of a new sputum culture prior to initiating treatment. While this could delay the effects of treatment, learners are more likely going to have more data on how to optimize the treatment approach. Future education should help clinicians understand the best approach, based on expert opinion and available evidence.

PERFORMANCE ASSESSMENT – CULTURE OR TREAT? BY ROLE

Case #1 continued: Two months later, the patient had an exacerbation and was treated with ciprofloxacin for two weeks. You see the patient in your office two weeks after this, and she presents with increasing cough, fatigue, and worsening shortness of breath. She reports good adherence with her medication regimen, which consists of ELX-TEZ-IVA, twice daily inhaled albuterol, dornase alfa, hypertonic saline, and tobramycin (every other month), twice daily chest physiotherapy, and oral azithromycin three times weekly. Her FEV1 at this visit is 50% predicted. Her most recent sputum culture from one month ago showed *Pseudomonas aeruginosa*.

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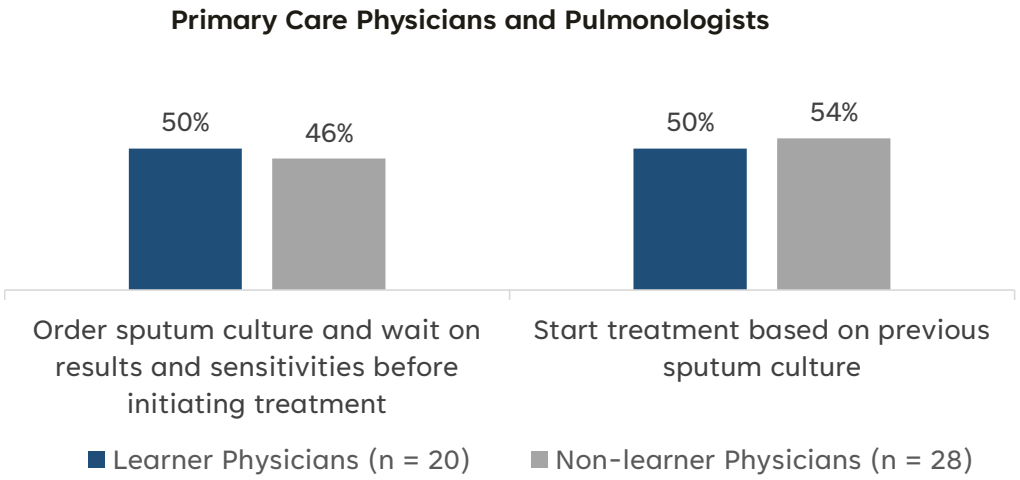
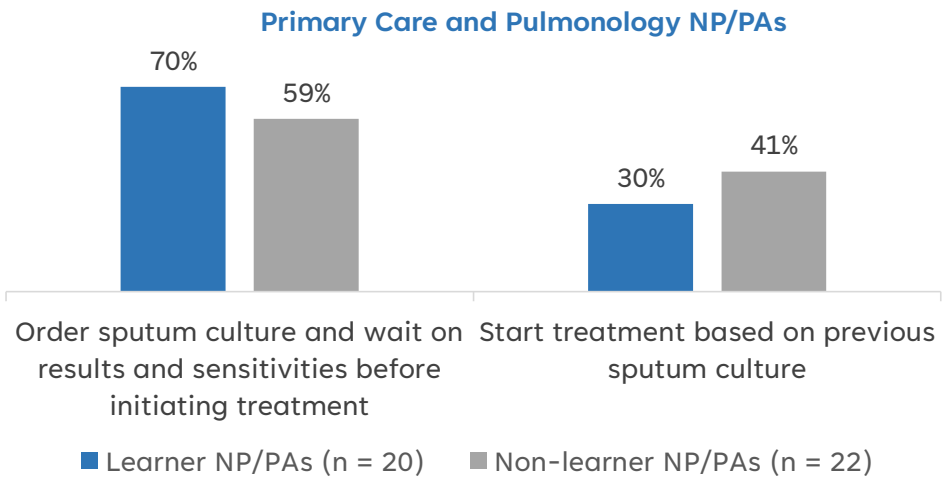




Learning Objectives Addressed:

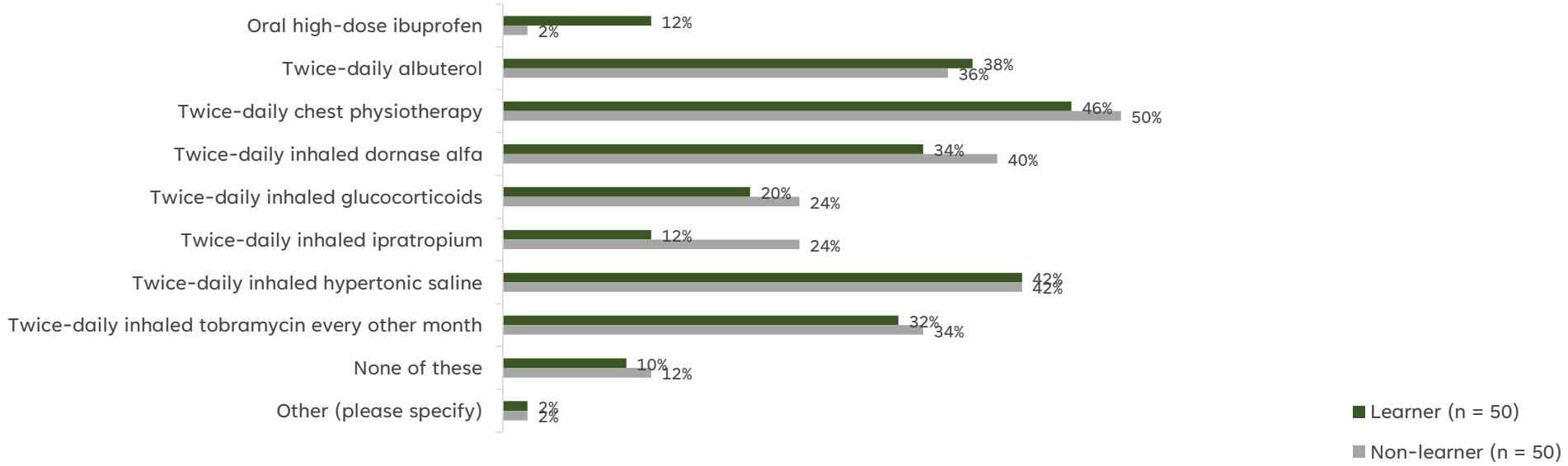
1

Clinicians are split on whether they would wait on updated sputum culture results prior to initiating treatment with a higher percentage of NP/PA learners indicating they would wait on the results than NP/PA non-learners.

PERFORMANCE ASSESSMENT – TREATMENT RECOMMENDATION

Case #2: A 21-year-old man with CF presents for a routine follow-up visit. He was diagnosed with CF when he was 2 years old due to failure to thrive. His current medications include pancreatic enzymes, elexacaftor-tezacaftor-ivacaftor (ELX-TEZ-IVA), and oral azithromycin 3 times weekly based on his chronic *Pseudomonas aeruginosa* infection. He is a college student and plays on a recreational basketball team. He performs chest physiotherapy about 3 times per week. He has been hospitalized once in the past for IV antibiotics when he was 7 years old and he has exacerbations of his CF about once a year. His FEV1 today is 75% predicted and his BMI is 21 kg/m^2.

Which therapies for lung disease would you recommend for this patient? (select all that apply)



Learning Objectives Addressed:

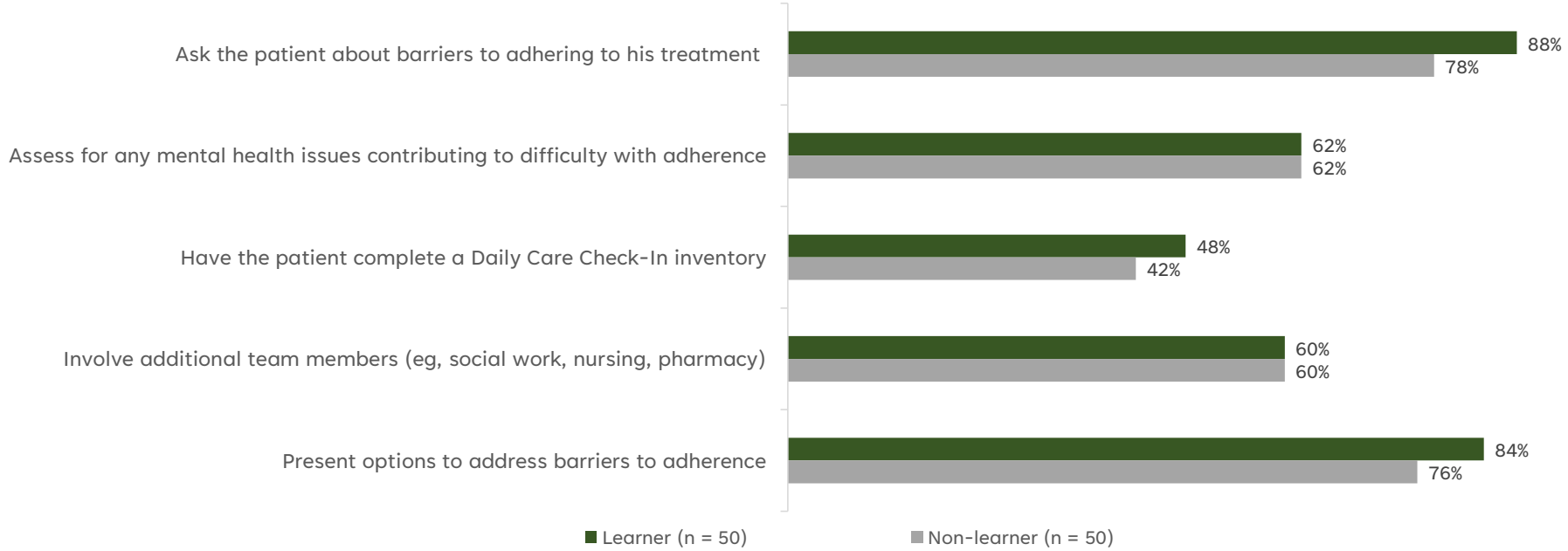
2

Based on the results of the SIMPLIFY study, this patient does not need twice daily dornase alfa and hypertonic saline. Continued reinforcement is needed to understand appropriate treatment considerations in this complicated space. The evidence from this trial as well as how it should be applied to specific patient types would be valuable in case presentations and problem-solving activities.

PERFORMANCE ASSESSMENT – IMPROVING ADHERENCE

Case #2 continued: The patient returns for routine follow-up in 3 months. Upon questioning, you determine that he is missing his ELX-TEZ-IVA dose twice per week on average.

How likely are you to do the following to attempt to improve this patient's adherence to his medication regimen?
(very → extremely likely)



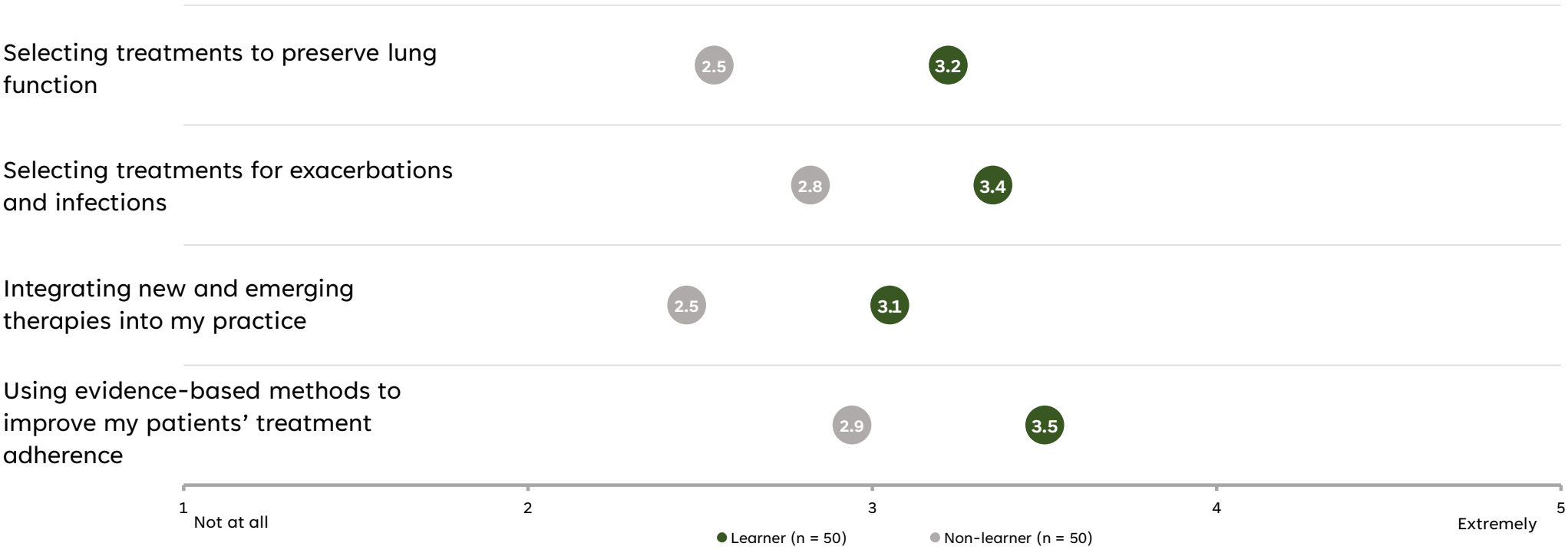
Learning Objectives Addressed:

3

Following engagement in the education learners express a higher likelihood than non-learners to ask the patient about barriers to adhering to treatment and a higher likelihood to present the patient with options to address barriers to adherence. Over half of clinicians indicate they are very or extremely likely to involve additional team members and assess for mental health issues that may contribute to difficulty with adherence.

PERFORMANCE ASSESSMENT – CONFIDENCE

Please rate your confidence in the following aspects of cystic fibrosis



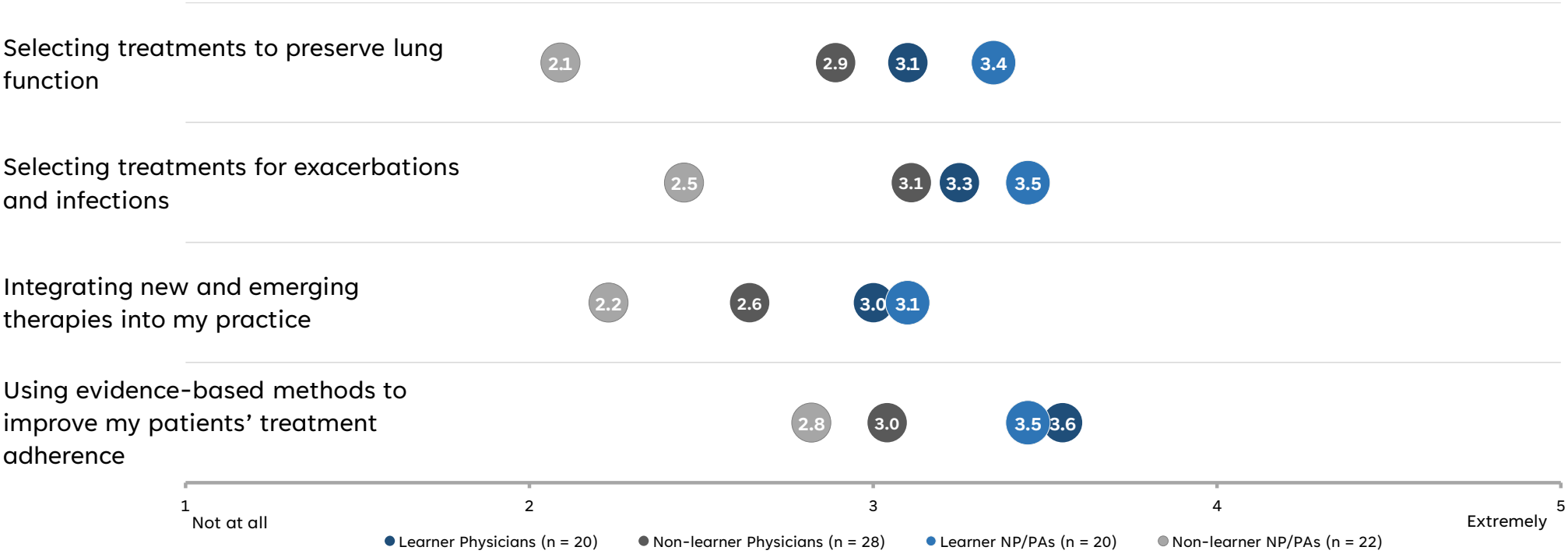
Learning Objectives Addressed:

1 2 3

At follow-up, learners indicated higher confidence than non-learners in selecting treatments to preserve lung function, to treat exacerbations and infections, integrating new and emerging therapies, and implementing evidence-based methods to improve patient adherence.

PERFORMANCE ASSESSMENT – CONFIDENCE BY ROLE

Please rate your confidence in the following aspects of cystic fibrosis



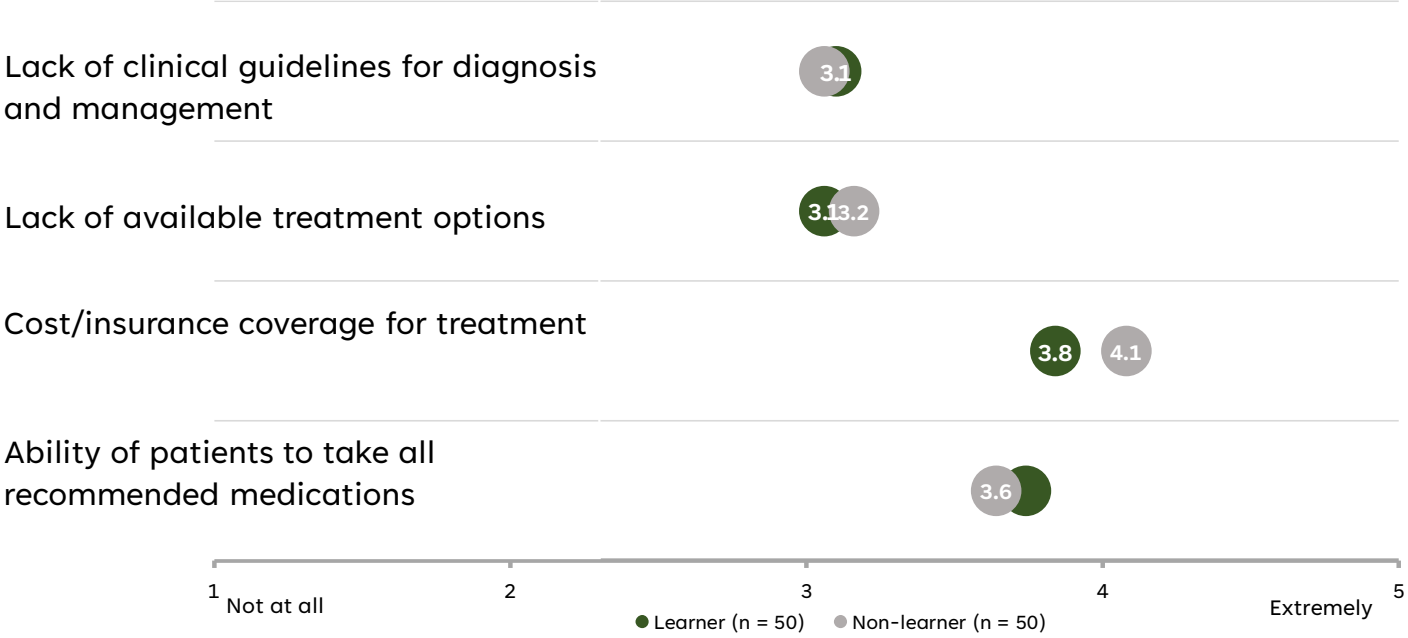
Learning Objectives Addressed:

1 2 3

When comparing physicians and NP/PAs to the non-learner controls, NP/PAs are much more confident than their non-learner counterparts, particularly in selecting treatments. Overall, the NP/PA confidence levels is higher than that of the physician group.

PERFORMANCE ASSESSMENT – BARRIERS

Please rate the significance of the following barriers to the optimal management of patients with cystic fibrosis.



Cost/insurance coverage is reported as a very significant barrier to optimal management of patients with cystic fibrosis by clinicians followed by the ability of patients to take all recommended medications.

What are other barriers to the optimal management of your patients with cystic fibrosis?


Family support


Access to care/ CoE

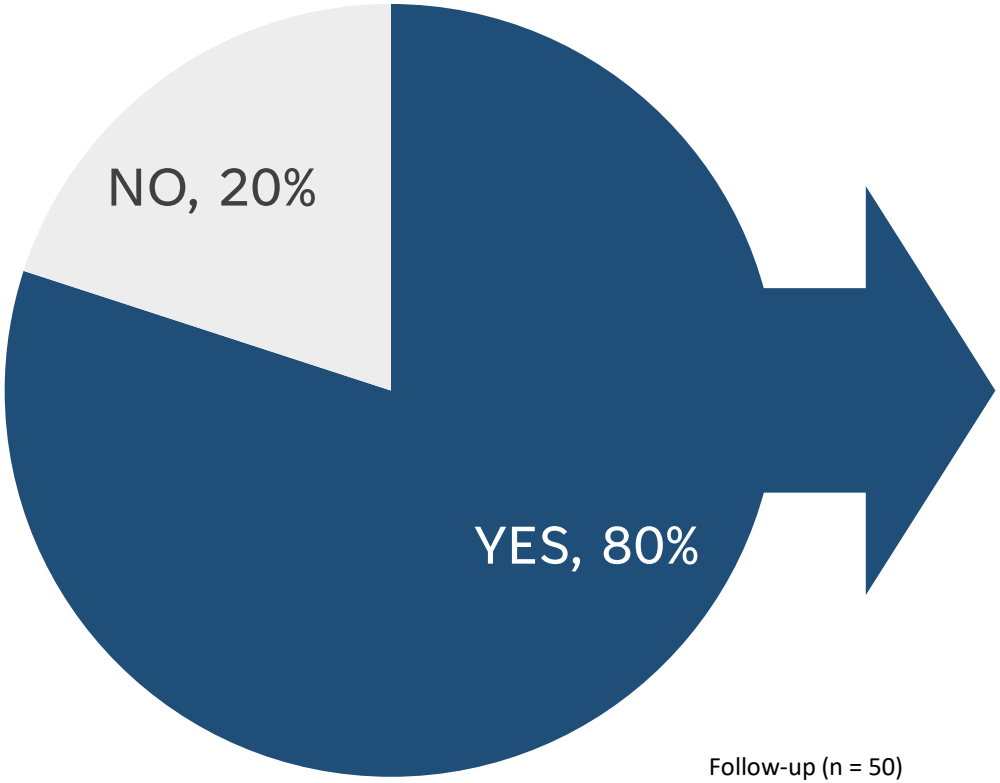

Time and travel for treatment


Multiple medications needed


Lack of clinical experience


Low patient/family education

Did the National Jewish Health program *Adherence: A Key to Successful Treatment of Cystic Fibrosis* address or help you overcome any of these barriers?



Follow-up (n = 50)

In what way?

- "Cost and available programs to assist with cost"
- "It helped me see better modalities and ways to address patient concerns."
- "Knowledge of new therapies"
- "I learned about different presentations and treatment options for CF."
- "Ongoing study, meta-analyses, and therapeutic updates"
- "More referrals out"
- "[I have] very few patients and [don't have] a protocol. Education helped me to gain more knowledge in treating CF patients."
- "Family resources"
- "Advanced therapy"
- "Able to get patient started on the appropriate medical regimen"
- "I am more familiar with standard of care"
- "Being able to provide patients with education and other resources to help them."

PERFORMANCE – NEW/SURPRISING?

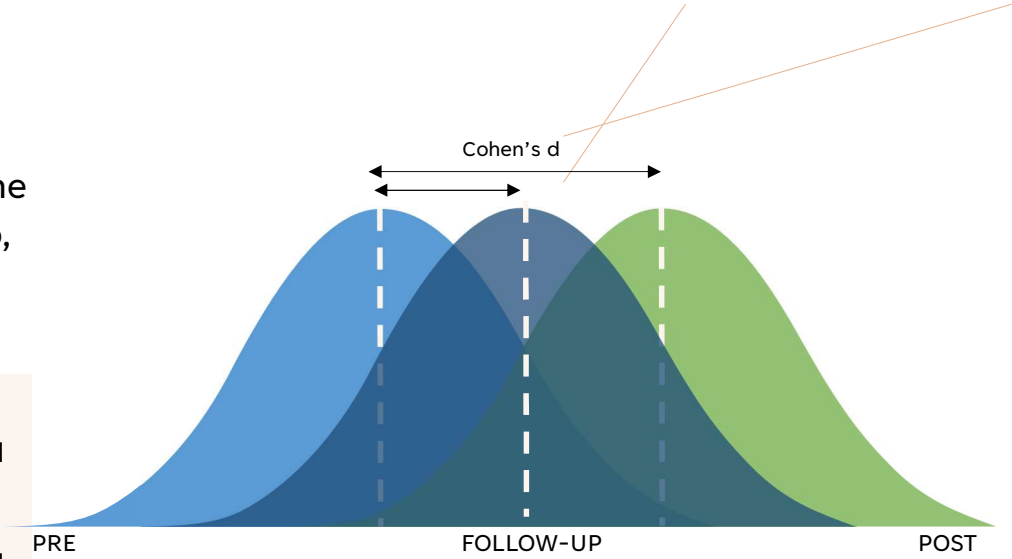
Did you learn anything in the program about CF that was new and/or surprising?

(open-ended question, direct quotes)

- “Treatment options to improve survival”
- “The availability of new treatment modalities”
- “The new tx's available and protocol changes from my basic science education years ago”
- “75% of the CME was new information; I found the pharmacotherapies the most interesting.”
- “More about other organs involved and the genetics”
- “It is always good to see what is in development and in the testing phases for future modalities.”
- “Discontinuation of dornase alfa/hypertonic saline”
- “All of it was new to me for the most part. It was slightly overwhelming as we barely touched on it in PA school.”
- “Most was new to me.”
- “Update on newer availabilities in treatment”
- “Yes- I [am] still going to send to specialist”
- “Treatment alternatives”
- “Yes, high dose medication use”
- “A general approach”
- “Medications and assistance available for CF patients”
- “The overall management of the patients”
- “Yes. Guidelines”
- “Most was new to me”

OVERALL EDUCATIONAL IMPACT

This education had a Cohen's d effect size of **1.27** comparing the pre- and post-education groups. Comparing pre- to follow-up, the education had an effect size of **0.55**.



INTERPRETATION OF COHEN'S D EFFECT SIZE

Assessment researchers often use general guidelines to help interpret Cohen's d – small (0.2), medium (0.5), and large (0.8).

With a Cohen's d of 1.27, 90% of the learner group will perform better compared to before their engagement in the education. For more information on interpreting Cohen's d, refer to https://rpsychologist.com/cohend/

The follow-up showed reinforcement of these knowledge gains, as well as key attitudinal shifts in the management of patients with cystic fibrosis. Specifically, learners were more likely than a non-learner control to discuss and present options to barriers to adherence with patients. Further, learners are more confident in their treatment selection, new therapy integration, and improvement of patient adherence.

While learners and non-learners reported similar barriers to care, 80% of learners indicated that the educational activity helped them overcome these barriers, including the provision of needed resources, protocols, and financial assistance programs.

Key Takeaways

- CF is a very complicated disease that deserves a specialist skill set for treatment
- The education confirmed the importance of adherence in CF disease management
- There are multiple ways to promote adherence
- Management of patients with CF is multifactorial and treatment options are continuing to evolve
- Application of new therapies into practice
- Prevention of treatment failure
- Discontinuation is possible in some instances and current modulation works
- The information of the newer therapies being developed and the potential to simplify and improve the lives of CF patients
- Have to find the right treatment for each patient
- Practical applications of the SIMPLIFY study
- The clinical trial data is a good start, but further data is still needed

Future Educational Needs

- In-depth education on treatment options
- Understanding management of adverse events that occur with CFTR modulator therapy
- Any new treatments on the horizon for those not candidates for current treatment
- The role of lung transplants for CF patients and gene therapy
- Stories directly from patients and what has worked for them
- Antibiotic treatments and duration
- Medications used to improve lung function

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Please contact CE Outcomes with any questions or to request further analyses of this educational activity.

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