

NTM Lung Disease: Patient Experiences & Preferences

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Disclosures

- No personal disclosures.
- NTMir organization disclosures:
 - Commercial support
 - Member of NTM Research Consortium



NTM Patient Experiences

- Long delays to proper diagnosis
- Long, burdensome treatment regimens
- Side effects, some permanent
- Symptoms including:
 - Severe cough
 - Extreme fatigue
 - Shortness of breath
- Unpredictability in day-to-day health and functioning
- Social isolation
- Social stigma



Survey:

Patient Preferences in Clinical Trials & Treatment Outcomes

- 57 questions
- Joint project with Spero Therapeutics
- Reviewed by internal and external staff and researchers, panel of five NTM patients
- Survey open March 6 – 25, 2019
- Circulated through NTMinfo.org, social media channels, blast emails, Social360 forum
- 465 respondents

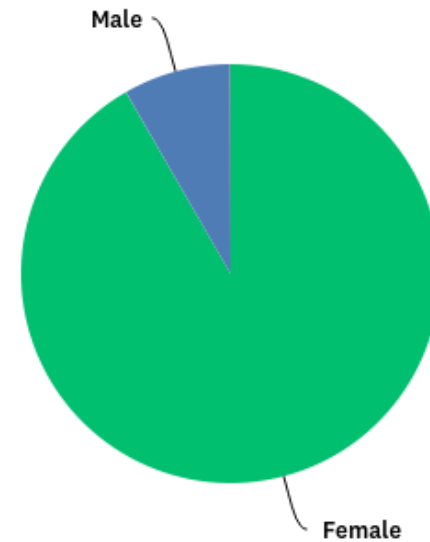
Respondent Age / Gender

Age

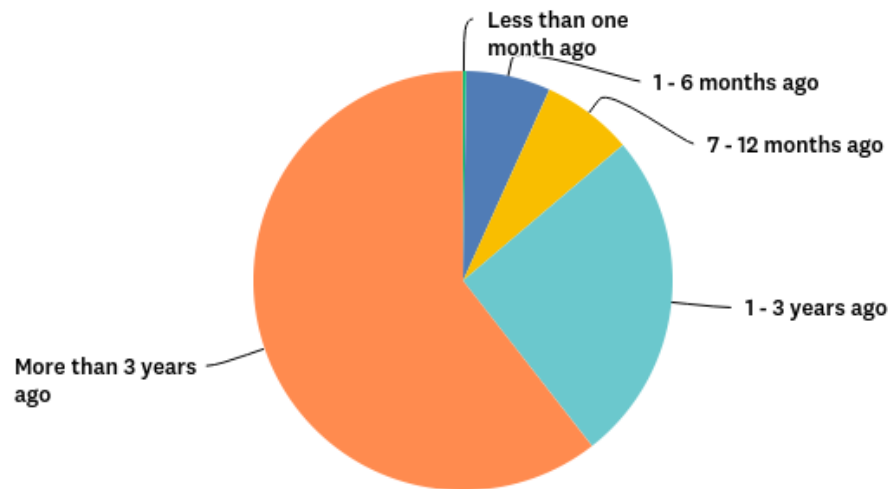
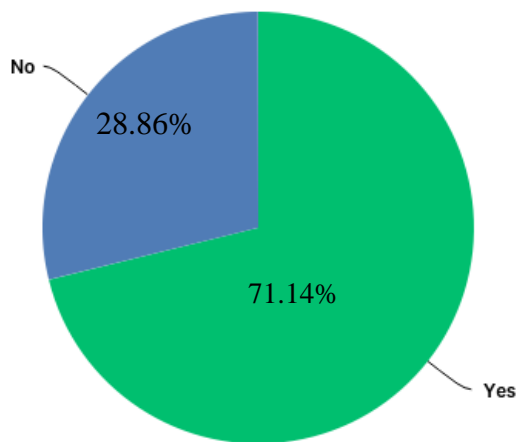
- Average: 65.9 years
- Range: 18-94 years

Gender

- Females: 92%
- Males: 8%



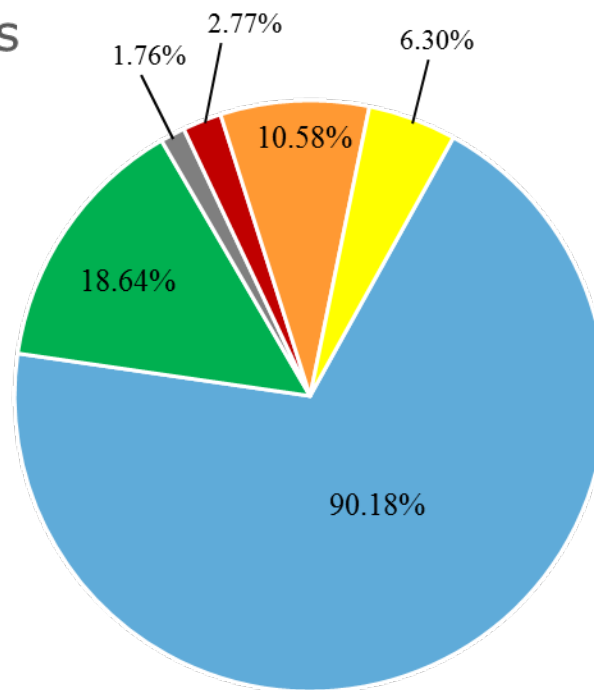
Current NTM Lung Infection



- 0.25% were diagnosed less than one month ago.
- 6.53%: 1-6 months ago.
- 7.04%: 7-12 months ago.
- 25.63%: 1-3 years ago.
- 60.55%: more than 3 years ago.

NTM Strains

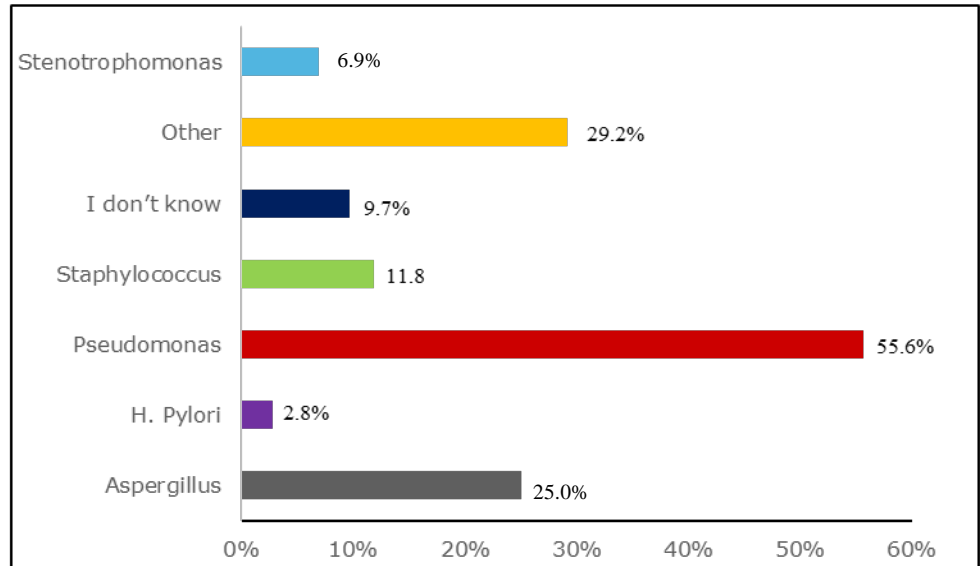
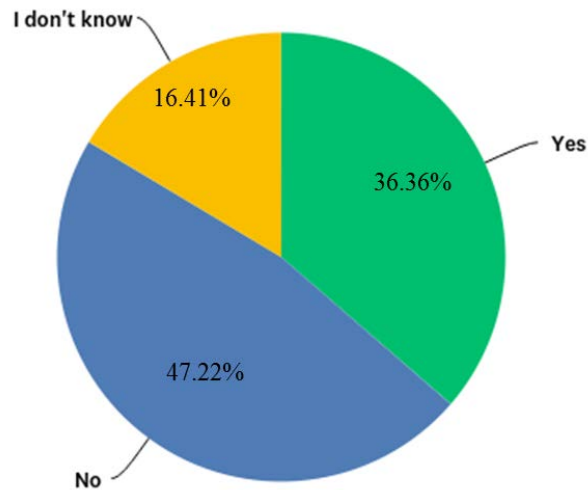
NTM Strains



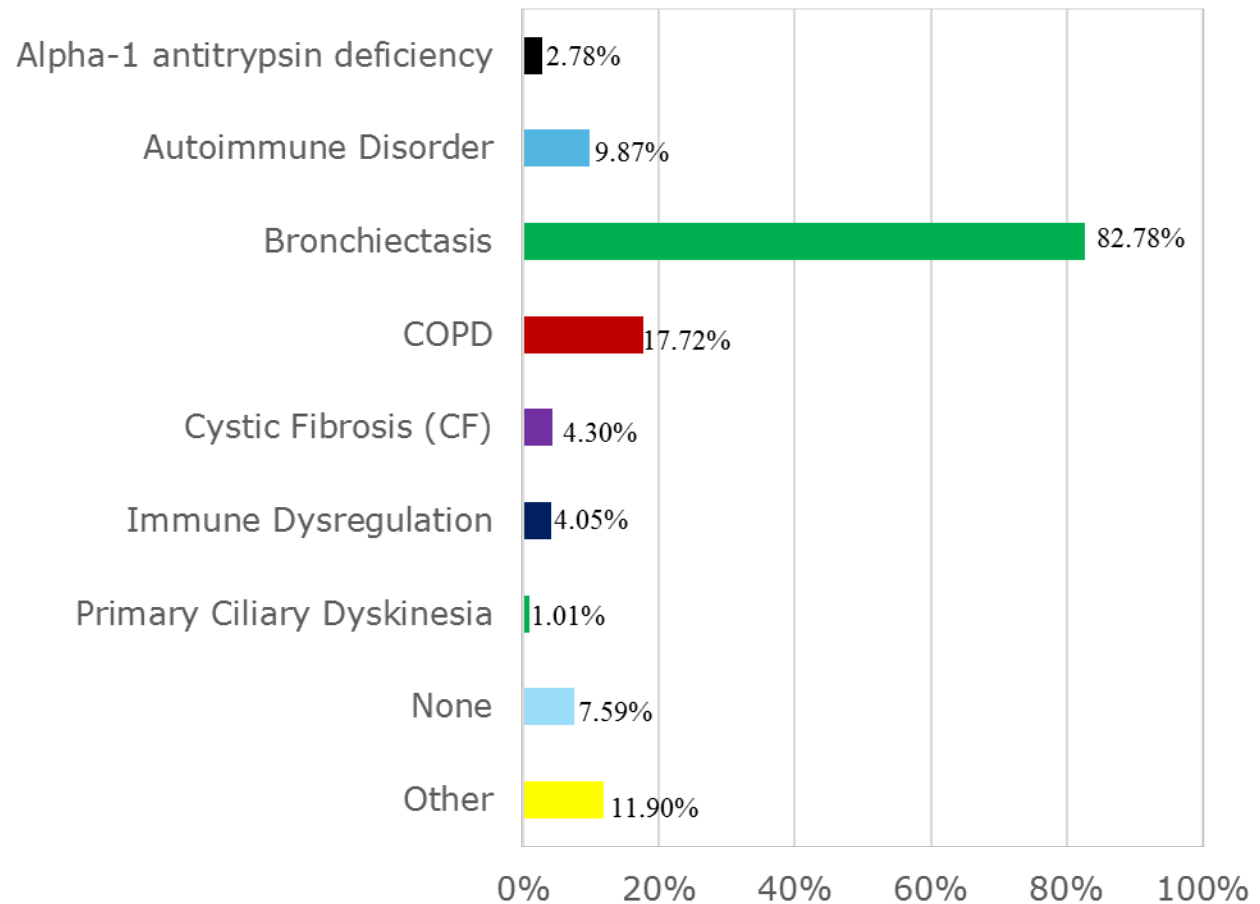
- M. Avium Complex
- M. Abscessus
- M. Kansasii
- M. Fortuitum
- Other
- I don't Know

Other Infections

Do you have other bacterial or fungal lung infections?

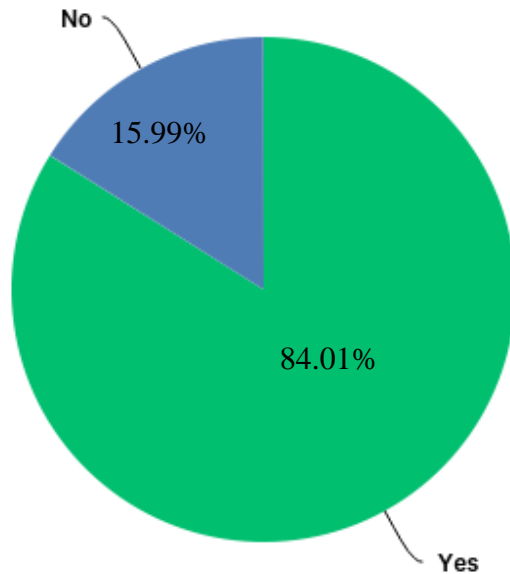


Comorbidities

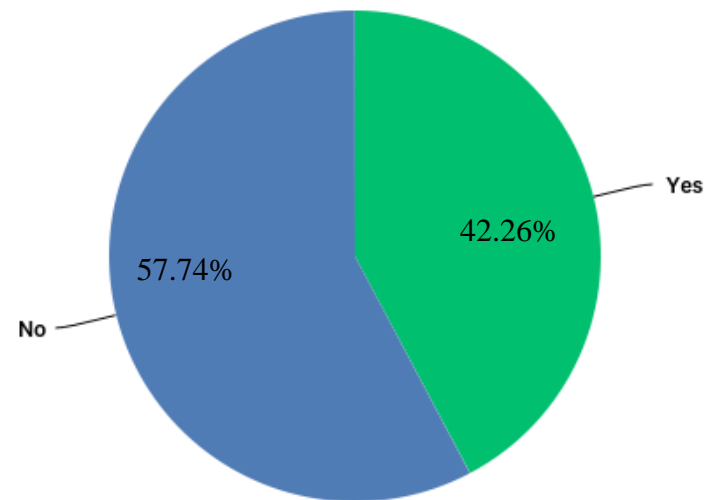


Antibiotic Treatment

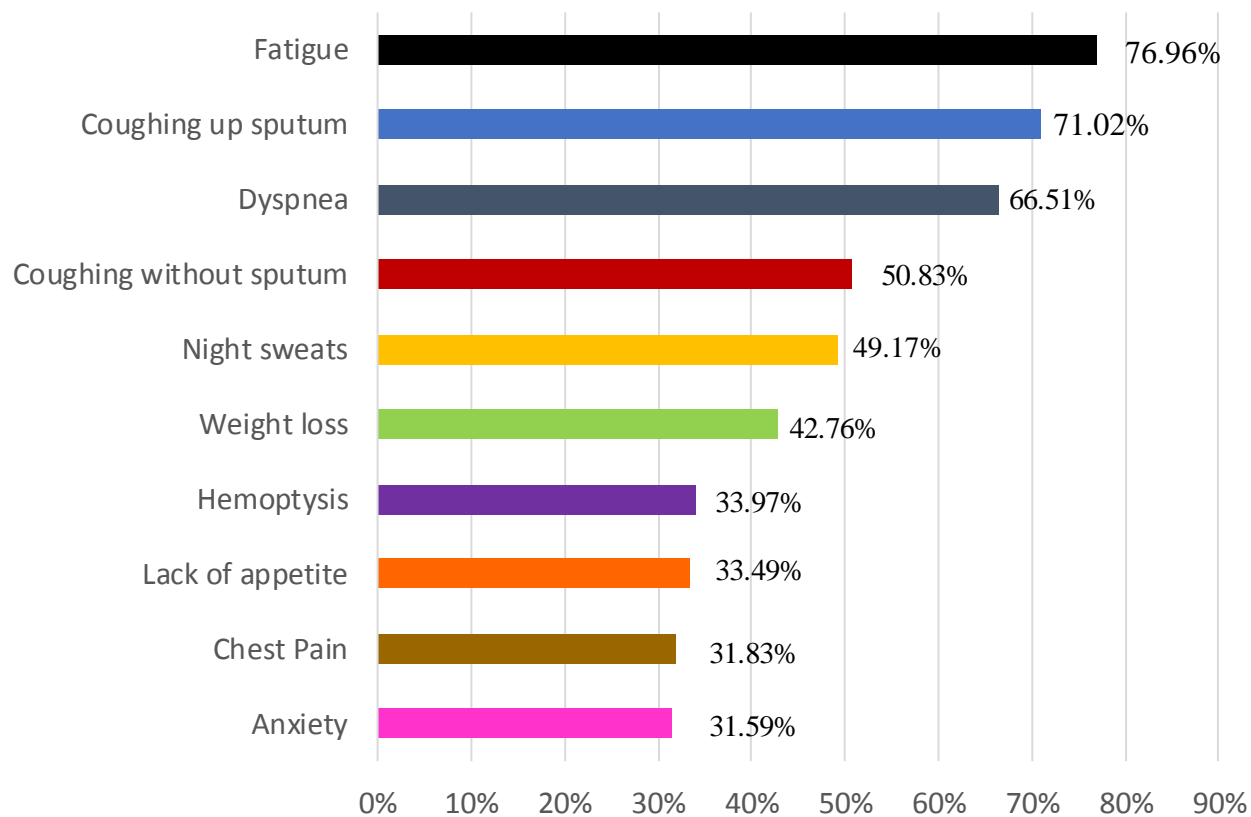
Have you ever been treated for your NTM infection with antibiotics?



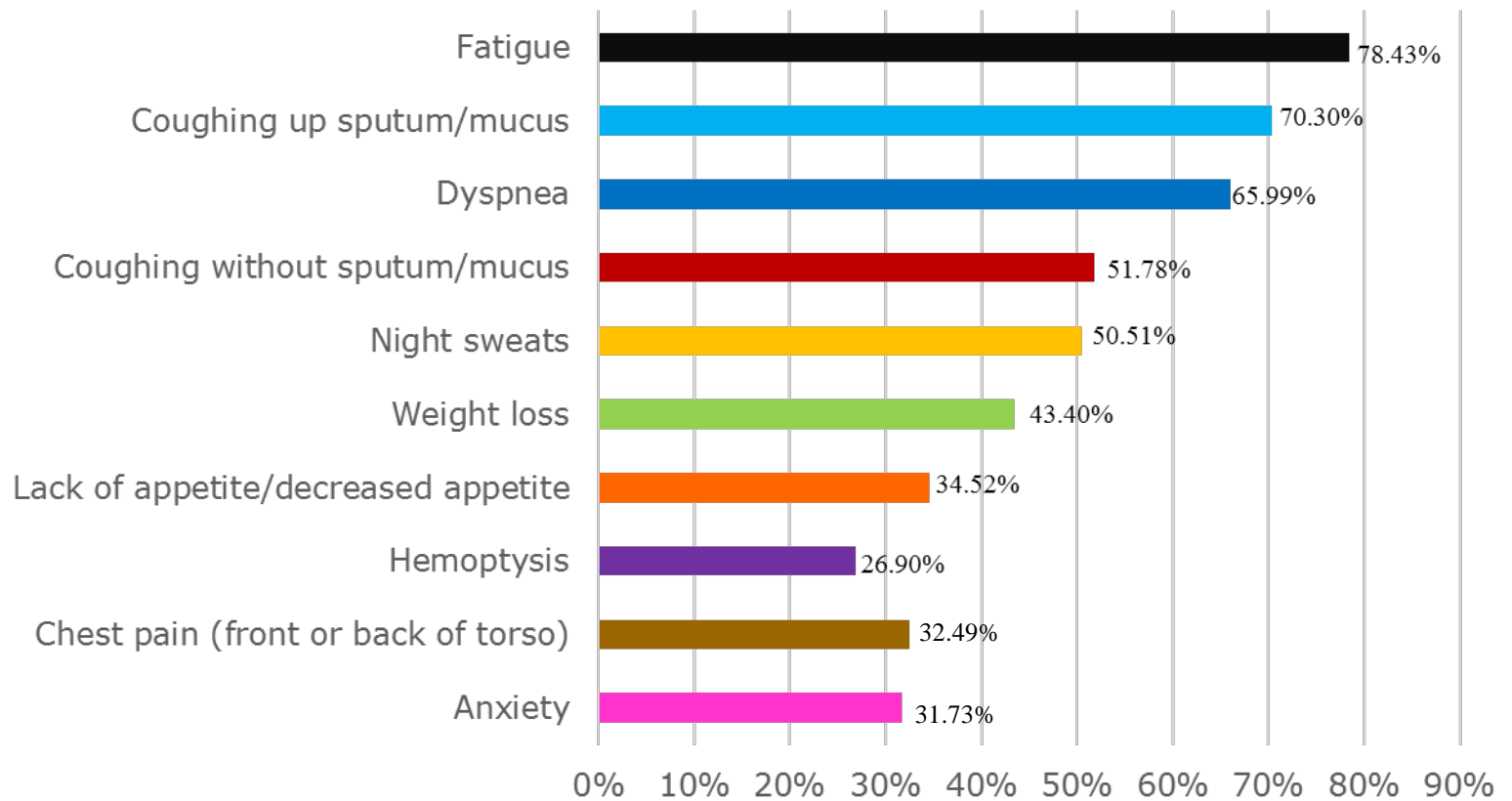
Are you currently on antibiotics for your NTM infection?



Top Symptoms Patients Experienced



Most Bothersome Symptoms





Most Bothersome Symptoms

“I cough every morning upon waking, so I don’t feel rested.”

“Coughing all the time with excessive mucous is a bother, interfering with everyday life.”

“The shortness of breath was frightening, I had never experienced this before.”

“Relentless cough leads to vomiting.”

“Superwoman can’t get much done if tired.”

“The shortness of breath was noticeable and affected my ability to do the things I was previously able to do.”

“I am so anxious I panic to the point of blood pressure going high.”

“Unable to function or do anything. No quality of life. You just exist from day to day. Week to week.”

“Coughing is exhausting. Coughing around others also makes them feel like you have a communicable disease so it can isolate you.”

“I prefer to sleep all night. Night sweats wake me up, I’m more tired the next day.”

“Because not being able to breathe sucks.”

Most Bothersome Symptoms – Impact

“It reduces the quality of my life.”

“Fatigue is a major problem as well as memory loss.”

“Fatigue makes it difficult to complete daily tasks.”

“The anxiety made me feel like I was dying.”

“I would sleep at night and wake up and be exhausted.”

“It’s embarrassing to have to cough up mucous and blood in front of clients, friends and even family.”

“People think you are contagious. You go from a “ball of fire” to a stay at home dud!”

“Caused shortness of breath and I would often throw up from coughing so much.”

“I have to plan activities based on whether it is a “good” day or not. I have to excuse myself from social gatherings to expectorate. I have to interrupt activities to take care of the mucus I generate.”

“Social isolation.”

“Mucous interferes with daily life!”

“How would you feel about starting your day when you feel so bad?”

Preferences in Treatment Outcomes

Outcome	# / Patients Indicating Preference
Improve quality of life	97
Increase energy/Less fatigue	84
Culture conversion	72
Reduce coughing	53
Improvement in dyspnea	42
Repair lung damage	28
Improve lung function	27
Reduce progression of disease	21
Reduce mucus/sputum	20
Reduce symptoms	14

“I hope it can be cured forever so that it never comes back.”

“Get rid of the bleeding, get rid of the mucous, get rid of the NTM.”

“Make my life more normal.”

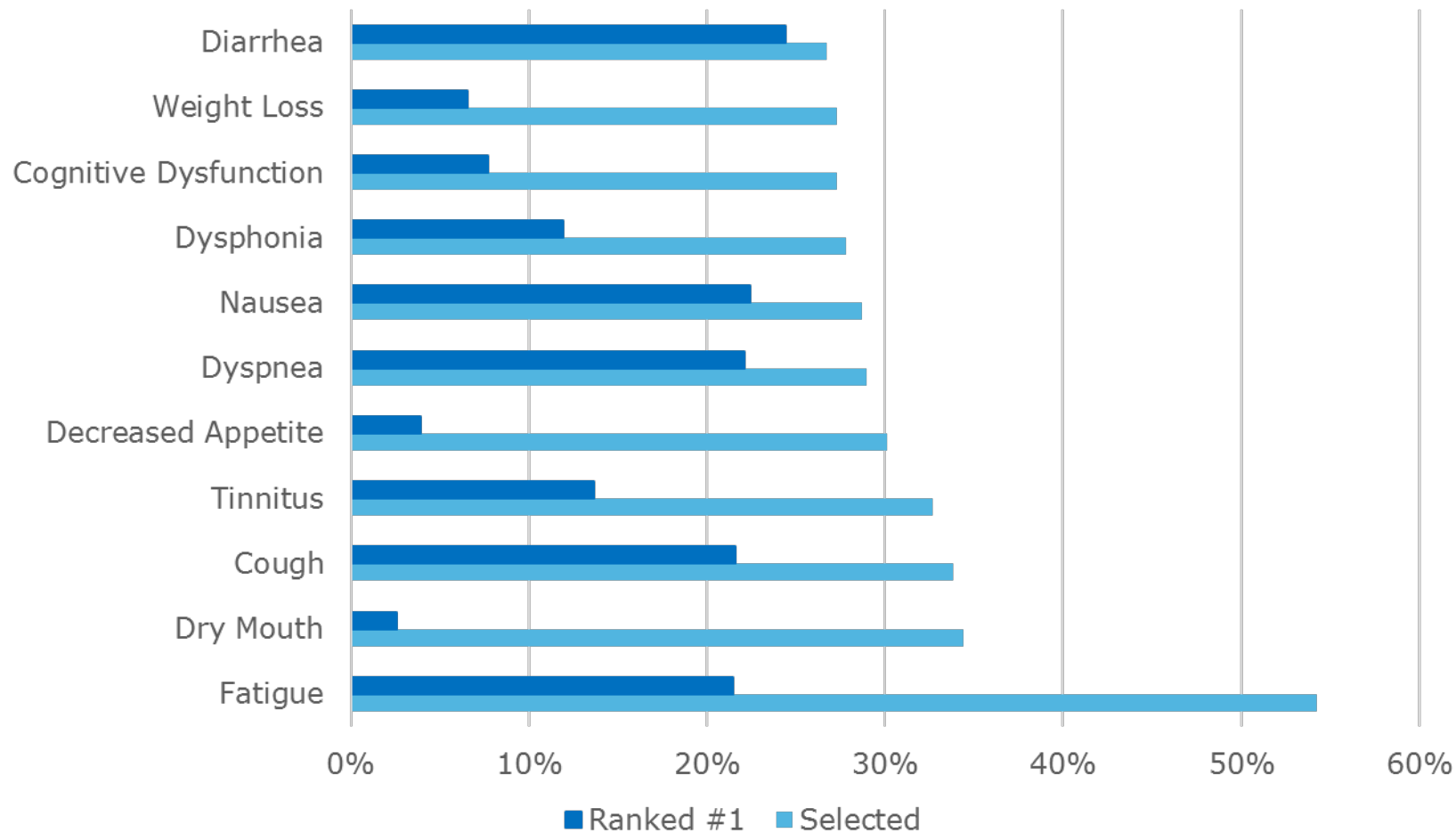
“Less coughing, less shortness of breath, less fatigue.”

“Prevent further lung damage.”

“Getting rid of it has improved my wellbeing.”

“I am most concerned with TOTAL lung health... getting this infection under control means I am less likely to have exacerbations that in turn require additional antibiotics in future.”

Side Effects of Antibiotic Treatment



Most Bothersome Side Effects – Impact

“Imagine not being able to breathe.”

“COUGH IS VIRTUALLY MY WORST PROBLEM!”

“Debilitating, embarrassing and socially frustrating.”

“Anxiety ruins everything about you. Not a normal life.”

“It’s so frequent it’s fatiguing and people want to know what is wrong. I call it my “forever” cough.”

“When I am nauseated I find it difficult to concentrate on anything else and just have to lie down.”

“I could wipe out an entire bus when coughing.”

“The rash severely itched and drove me crazy.”

“Shortness of breath makes you feel weak and tired.”

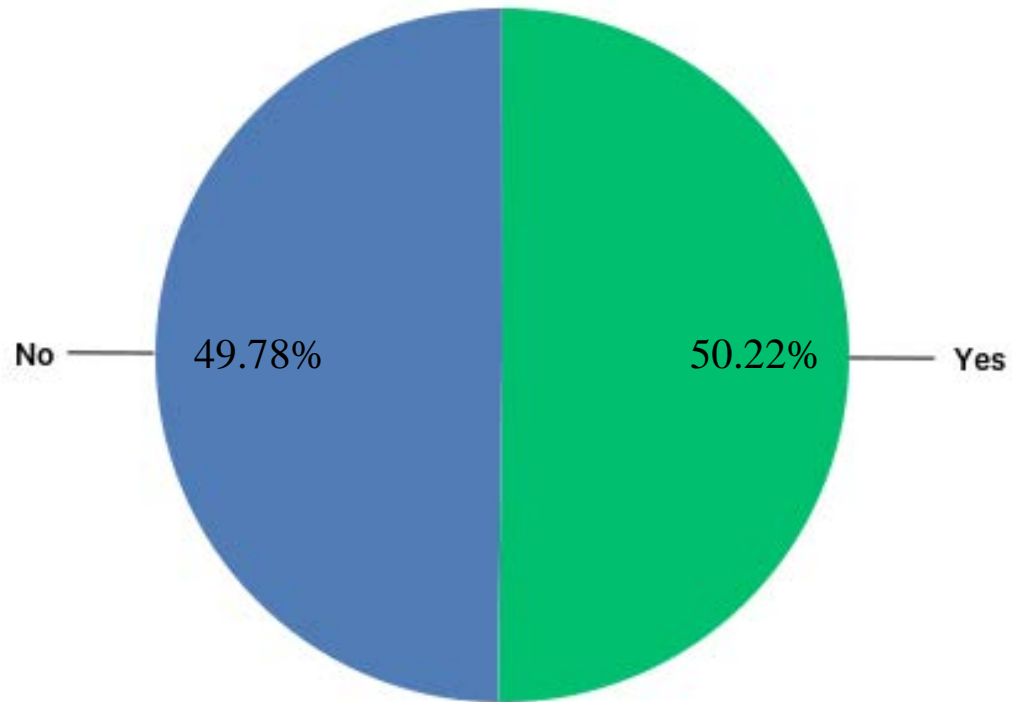
“I lose my voice which makes it difficult to communicate and very difficult to do my job as a telephonic nurse case manager.”

“CAUSED FATIGUE.”

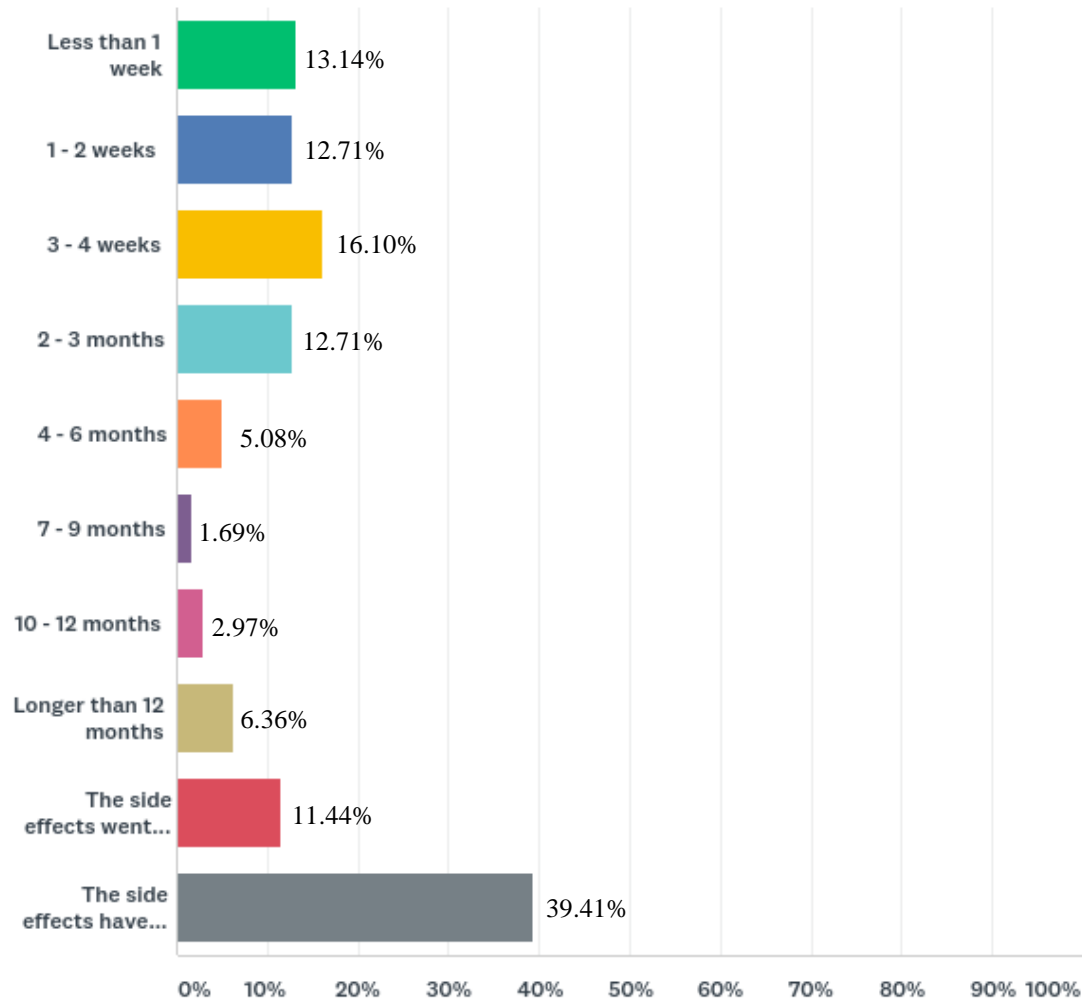
“Brain fog is affecting my ability to do my job.”

“Who wants hearing loss?!!”

Culture Converted Due to Treatment?



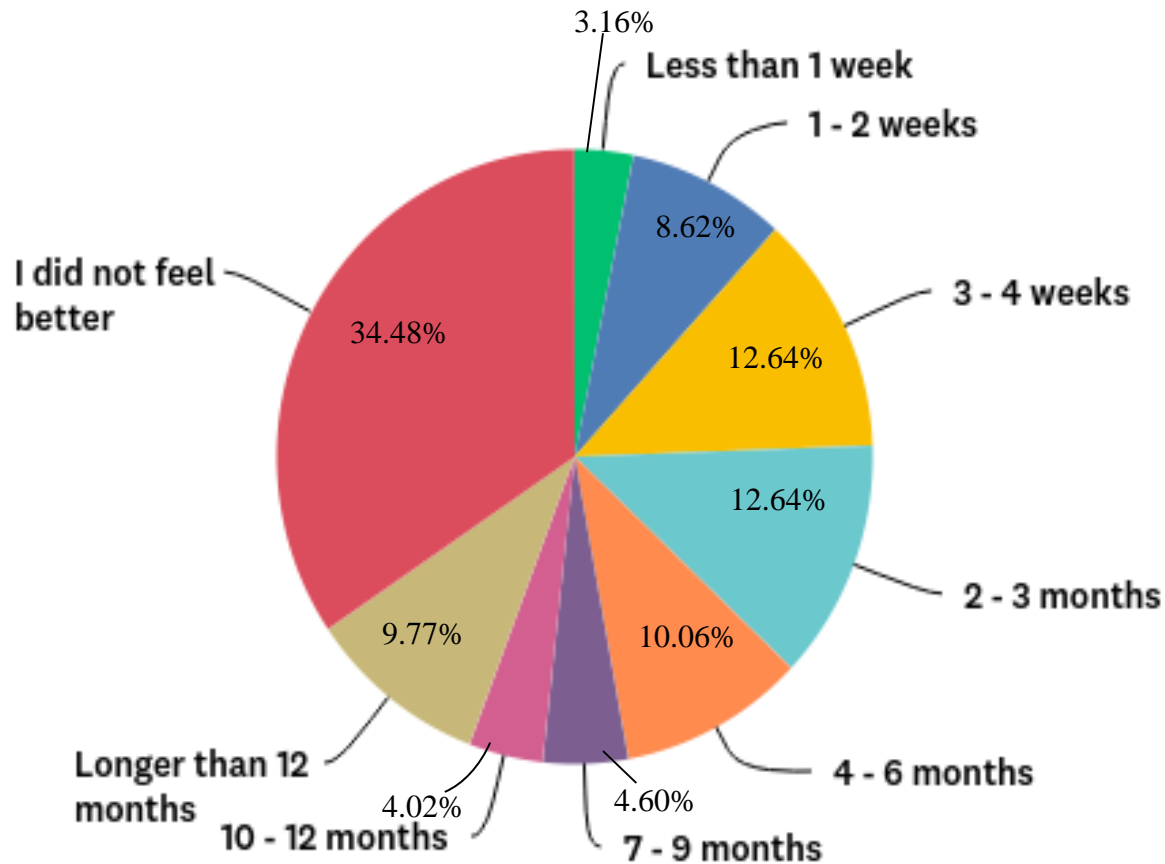
“How long after you stopped or completed treatment did the side effects subside?”



Duration of Side Effects

Side Effect	Went Away During Treatment (# Reporting)	Did Not Go Away (# Reporting)
Cough	11	16
Nausea	8	3
Fatigue	6	15
Diarrhea	3	3
Dysphonia	2	2
Dry Mouth	2	1
Pain	2	10
Dyspnea	2	6
Sputum Production	2	4
Vision Loss or Changes	2	6
Tinnitus	1	20
Cognitive Dysfunction	1	5

“How long after you began treatment did you begin to feel better?”



Improvement of Symptoms

Symptom Improvement	# Reporting Improvement
Reduced or no cough	79
Less fatigue	63
Reduced or no sputum	45
Reduced or no dyspnea	38
No fever	17
No night sweats	17
Reduced or no hemoptysis	13
Culture Conversion	9
Weight gain/no weight loss	9
Reduced chest tightness or congestion	9
Improved PFT's	8
Reduced or no chills	7
Increased appetite	7
Improved sleep	7
Decreased number of exacerbations (bronchitis, pneumonia)	6

“What bothers you most about your disease?”

“Until very recently, the lack of drugs specifically developed to treat NTM. Having an orphan disease is tough.”

“Not being able to hike, swim, snorkel due to shortness of breath.”

“COUGHING.”

“Tiredness.”

“Shortness of breath.”

“Not being able to breathe and coughing, particularly in the morning. I feel like I’m drowning in my mucous.”

“The cough and the mucus and the fatigue.”

“The unknown.”

“What bothers me most is the irreversible damage done to my lungs.”

“It inhibits my ability to enjoy living.”

“Fatigue is the hardest symptom.”

“Fatigue, coughing, lack of sleep.”

“It is with me daily. It is chronic. It is incurable. It has damaged my lungs permanently.”

“If your treatment could change one thing about your NTM lung disease, what would you want that one thing to be?”

Outcome	# / Patients Indicating Preference
Culture conversion	124
Reduce coughing	38
Increased energy/Less fatigue	35
Improvement in dyspnea	27
Shorter treatment regimen	23
Reduce mucus/sputum	20
Eliminate/reduce recurrence of infection	20
Fewer or no side effects	16
Repair lung damage	14
Improve lung function	12

“Maybe a medication that would reduce Bronchiectasis or reduce cavities?”

“With improved lung function I would be able to do the things I love again.”

“The results I have been seeing already, coughing less and having more stamina and energy.”

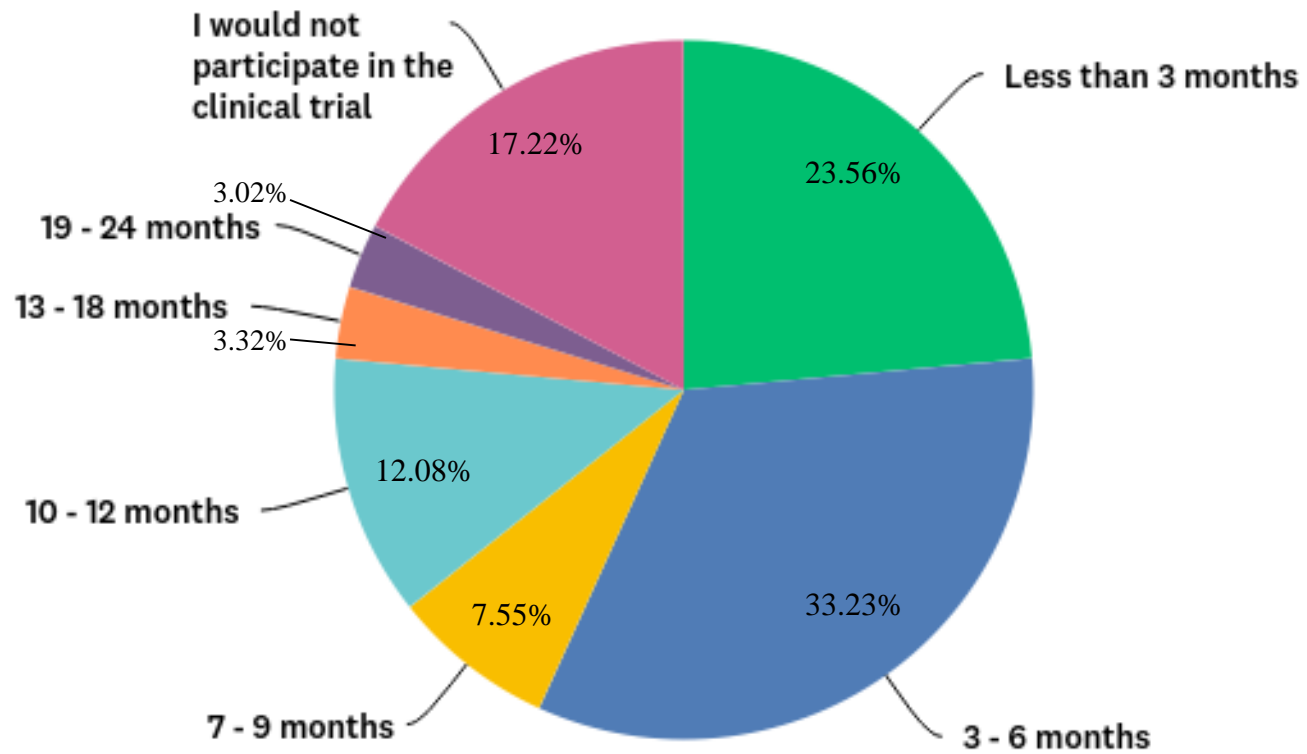
“I wish there was a treatment to minimize the permanent damage done to my lungs.”

“Mucus production is most bothersome.”

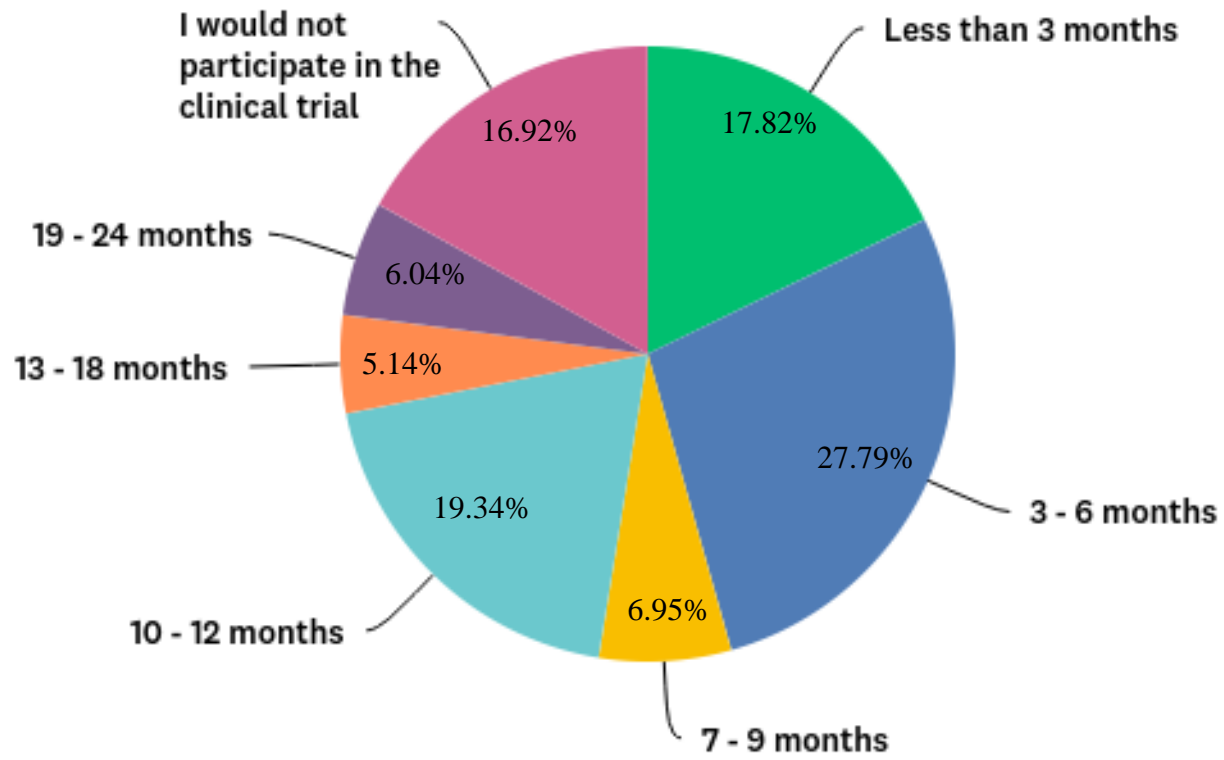
“What else? A CURE!”

“Complete healing without the probability that it will be reinfected in a short time.”

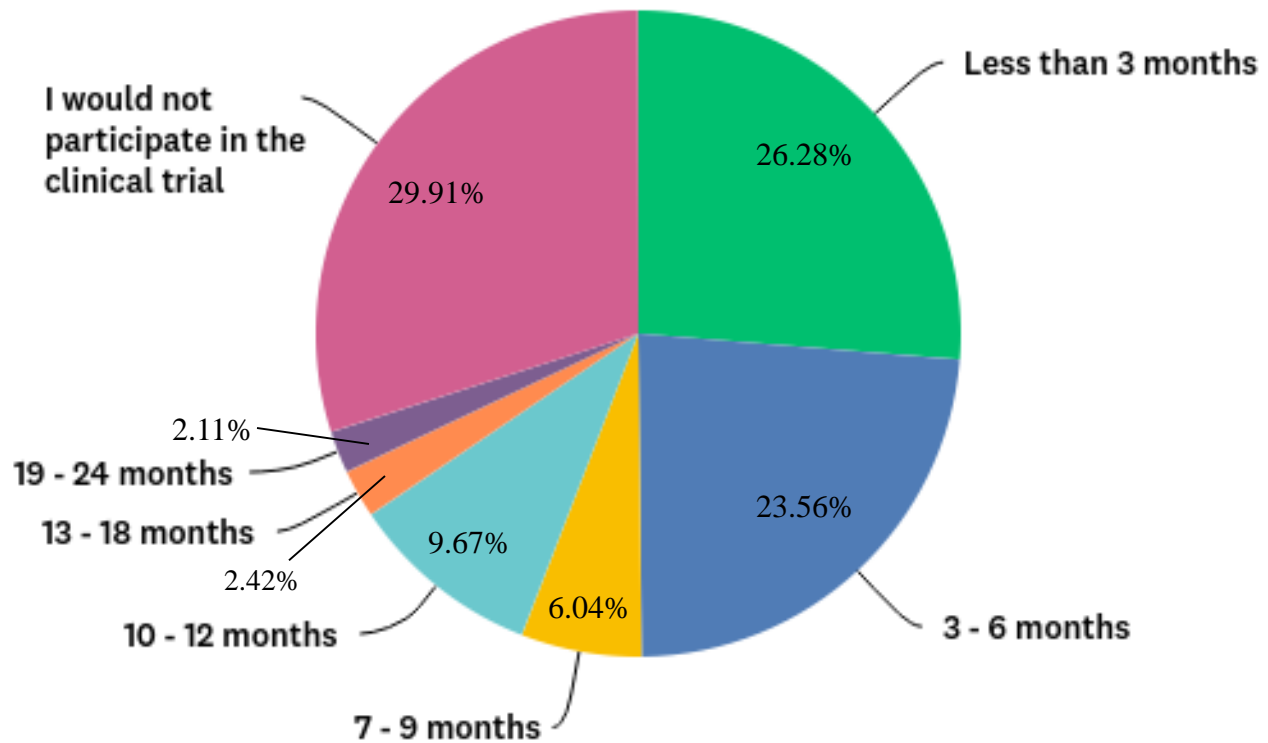
RCT / Treatment-Naïve: IND or Placebo



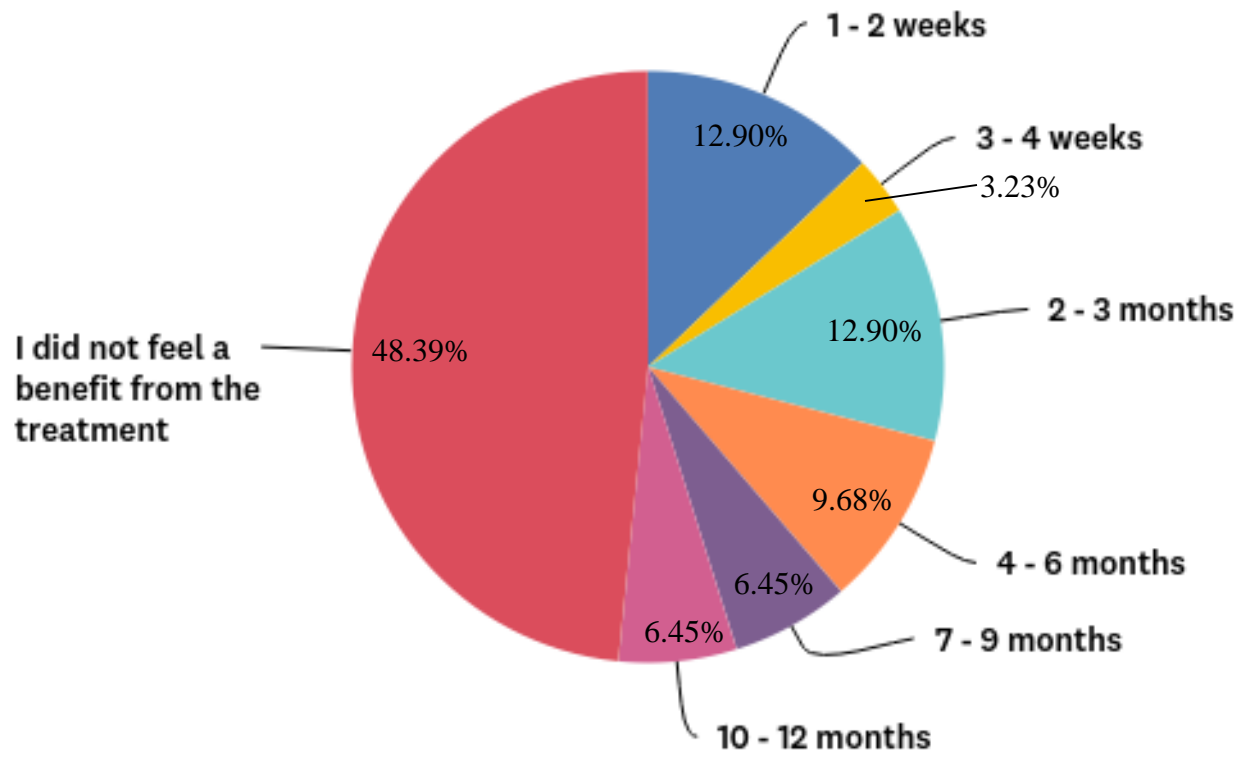
RCT / Refractory: SOC + IND/Placebo



RCT / Refractory: SOC Replaced with IND or Placebo



Length of Time to Improvement in Clinical Trials



Patient Experience in Clinical Trials:

Reported improvements (benefits) after investigational new treatment started

Reported Improvement(s)	#/Patients Reporting Improvement
Reduced coughing	6
Increased energy/Less fatigue	4
Improvement in dyspnea	4
Increased ability to exercise/improved 6MWT	3
Reduced mucus/sputum	2
General health improvement	2
Reduced hemoptysis	1
Weight gain	1
Hope	1



Patient Experience in Clinical Trials: *Reported improvements (benefits) after investigational new treatment started*

“It wasn’t until I ceased the LAI. 1 to 2 weeks after. I felt the best I’d felt in years.”

“Energy, no cough, weight gain, better 6mw.”

“I felt like I could breathe a little easier, my random low grade fevers stopped, and I was able to sleep better.”

“Could breathe deeper.”

“Nothing immediately, after I cleared I got more energy.”

“Greater lung capacity on exertion, less coughing up gunk.”

Patient Experience in Clinical Trials:

First reported improvement (benefit) after investigational new treatment started

Reported Improvement	#/Patients Reporting Improvement
Cough	5
General health/energy/fatigue	4
Dyspnea	3
Fever	1
Exercise	1
Congestion	1
Chest pain	1
Hemoptysis	1
Hope	1

Patient Experience in Clinical Trials:

First reported improvement (benefit) after investigational new treatment started

“No cough, wheezing, chest pain, fatigue.
I felt great.”

“Cough and massive sputum production stopped.”

“I didn’t feel as sick anymore.”

“More energy.”

“The random fevers stopped.”

“Easier to exercise.”

“Breathing better.”

“Easier breathing.”



Acknowledgements

- NTM patients
- Stephanie Yunis, NTMir
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THANK YOU