



EMBARC

The European Bronchiectasis Registry



ELF EUROPEAN
LUNG
FOUNDATION

Bronchiectasis and Non-Tuberculous Mycobacterial (NTM) lung infections Patient survey

Final report
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Introduction

This survey is part of the scope of work led by EMBARC developed in collaboration with the European Lung Foundation (ELF) and their bronchiectasis patient advisory group.

The aim of the survey was to find out about the challenges encountered during the evaluation and treatment of NTM-PD, to help healthcare professionals improve how they deal with this condition.

The diagnosis and decision to treat NTM-PD are often challenging and require multiple assessments, which may include many sputum (mucus) samples and sometimes bronchoscopies. It may also take a long time until a decision to treat is made. The treatment is also lengthy, and the drugs used may come with side effects.

The survey asked people with bronchiectasis, both with and without NTM-PD; and people with NTM-PD infection (current or past) with or without a diagnosis of bronchiectasis to complete the survey.

This report has been compiled by ELF staff based on the survey findings.

Short summary of survey results

Of the 347 respondents who were eligible for data analysis, 85% were female and 51% were between 51 and 70 years of age. The level of awareness of NTM before taking the survey was 60%; with 65% of respondents having been tested.

Of the 152 respondents who had experience of NTM, the most common species found in their sputum was *Mycobacterium avium* or *Mycobacterium intracellulare* (MAC or MAI).

The top three challenging issues for those diagnosed with NTM-PD were rated as 'Feeling tired', 'Cough' and 'Exacerbations'. Respondents also highlighted the impact of side effects, anxiety around dealing with their condition as well as the impact on their quality of life.

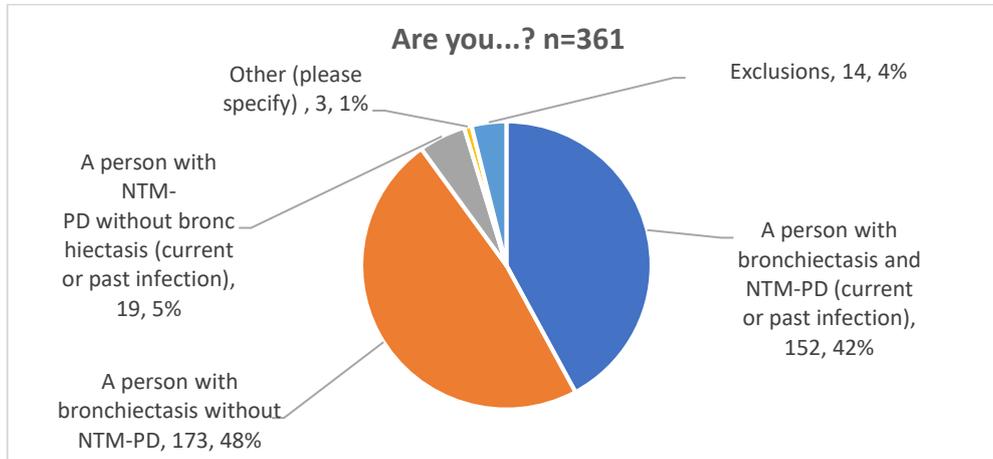
All of the issues needing attention to improve management of NTM-PD were rated between 'Important' and 'Very important' when all responses were averaged and the top three issues were: 'Finding methods to prevent NTM infection', 'Finding drugs for NTM-PD that have fewer side effects' and 'Finding drugs that are more effective in treating NTM-PD'. Many respondents highlighted the urgent need to raise awareness of NTM among all healthcare professionals including local doctors and to find ways of preventing re-infection.

The most difficult aspects to manage NTM-PD were identified as: 'Long duration of treatment'; 'Worries over diagnosis and treatment of NTM-PD' and 'Side-effects of drugs'.

Additional important points raised included the concern over lack of knowledge, the need for faster diagnosis, access to NTM-PD specialists and more research into prevention.

A. About the respondents

1. There were 361 respondents to the survey and the chart below shows how they identified themselves with 48% a person with bronchiectasis without NTM-PD and 42% a person with bronchiectasis and NTM-PD (current or past infection):



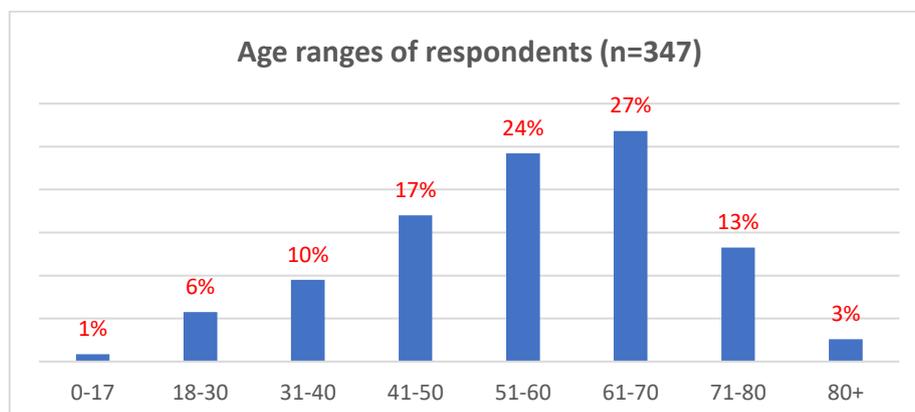
Exclusions: Of the 361 survey responses, there were 14 exclusions due to respondents not meeting the criteria of having bronchiectasis and/or NTM-PD. **This left a total of 347 responses considered for analysis.**

2. Respondents were located in the following countries:

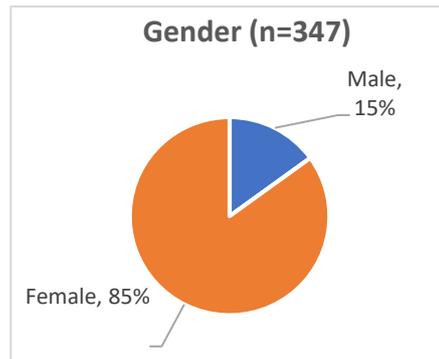
Afghanistan (n=2), Argentina (n=2), Australia (n=11), Austria (n=1), Azerbaijan (n=1), Belgium (n=4), Brazil (n=1), Canada (n=9), Chile (n=1), Finland (n=1), France (n=7), Germany (n=38), Greece (n=2), Iraq (n=1), Israel (n=4), Italy (n=96), Japan (n=2), Kazakhstan (n=1), Mexico (n=1), Netherlands (n=2), New Zealand (n=1), Peru (n=1), Poland (n=2), Portugal (n=3), Serbia (n=1), Spain (n=9), Sweden (n=1), Switzerland (n=3), Tunisia (n=1), Ukraine (n=2), UK (n=59), USA (n=76), Venezuela (n=1)

The countries where most respondents live are: Italy (27%), USA (21%), UK (16%) and Germany (11%).

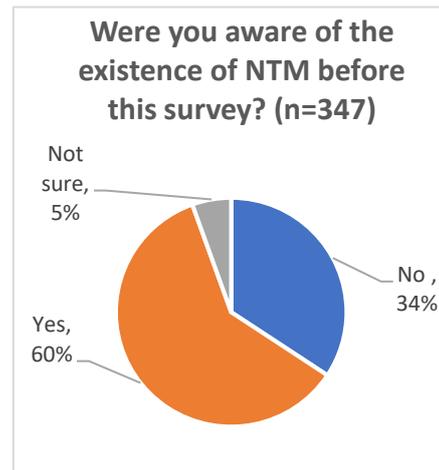
3. Age range of respondents is shown below; most being between the ages of 51 and 70:



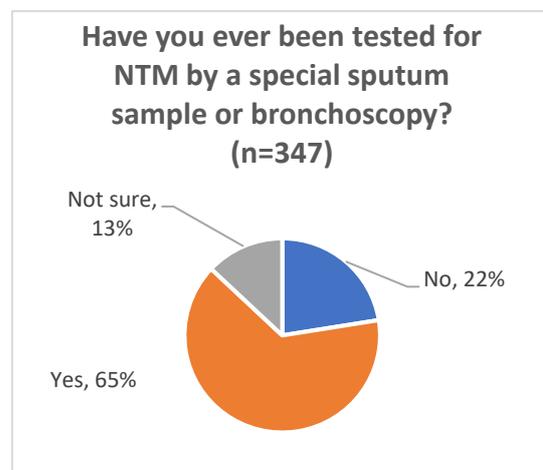
4. The gender of respondents is shown in the chart with majority being 'Female' (85%):



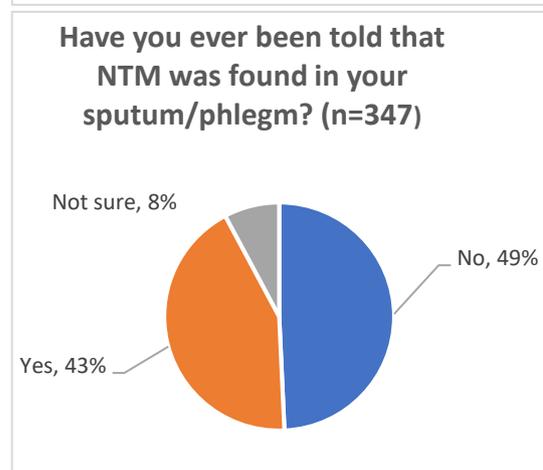
5. Respondents were asked about their awareness of NTM with 60% aware of NTM before taking this survey:



6. Respondents were asked if they had ever been tested for NTM and 65% had been tested:



7. Respondents were asked if they had ever been told that an NTM had been found in their sputum and 43% stated 'Yes':

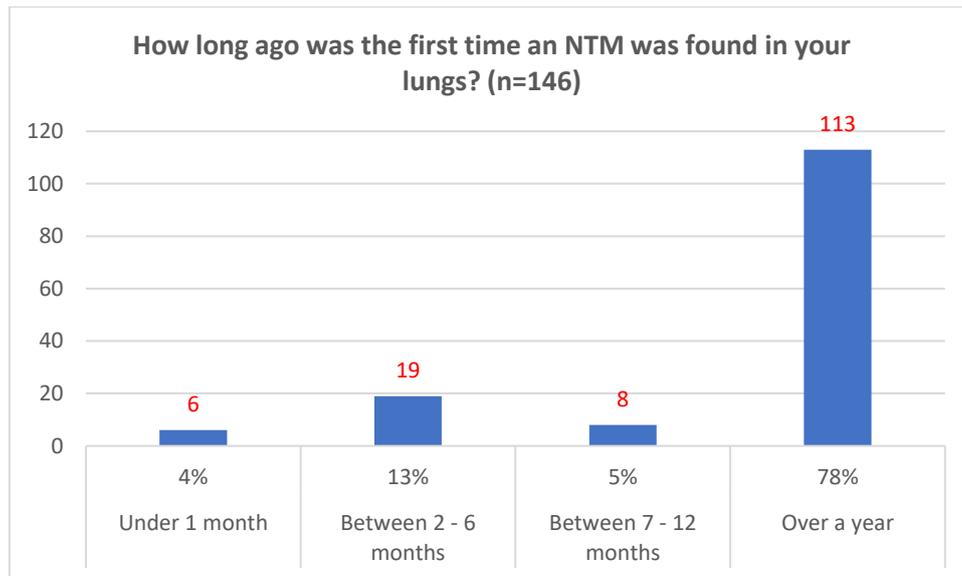


B. Your experiences of NTM

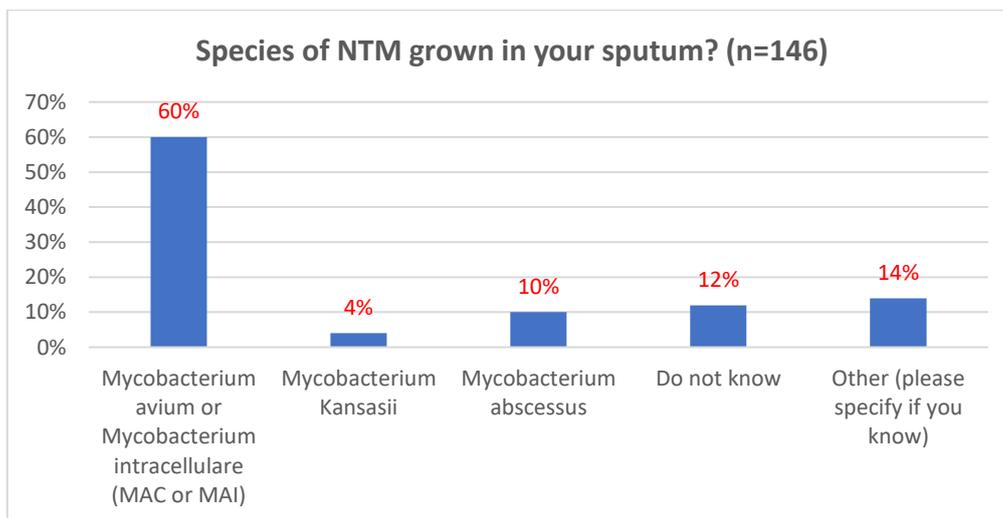
For this section of the questionnaire a **total of 152 respondents** were eligible to answer these questions as they had indicated that they had either been tested for NTM or been told that an NTM was found in their sputum.

NTM type and treatment

8. Respondents were asked how long ago was, the first time an NTM was found in their lungs with 78% stating it was 'Over a year':

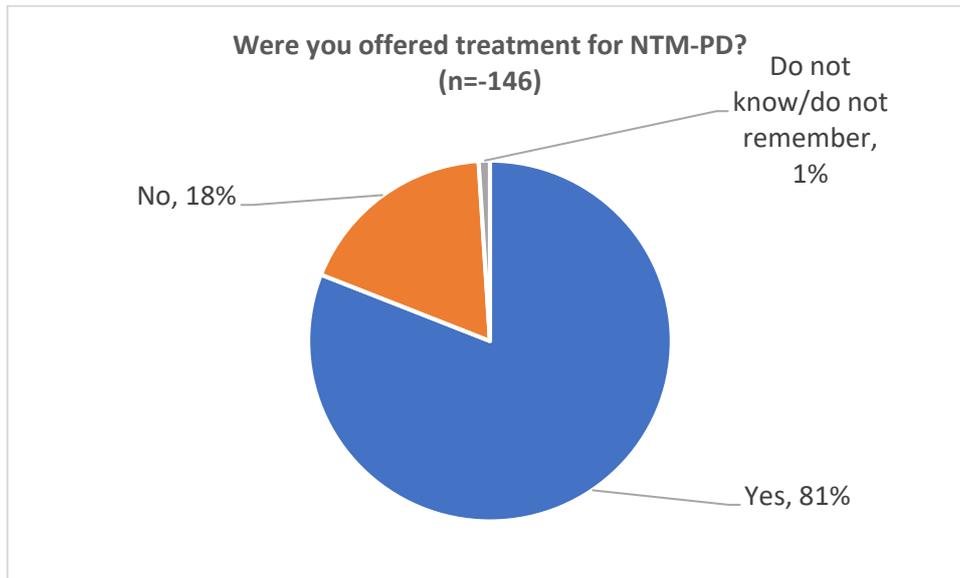


9. Respondents were asked about the type of species of NTM found with the majority (60%, n=87) stating Mycobacterium avium or Mycobacterium intracellulare (MAC or MAI):



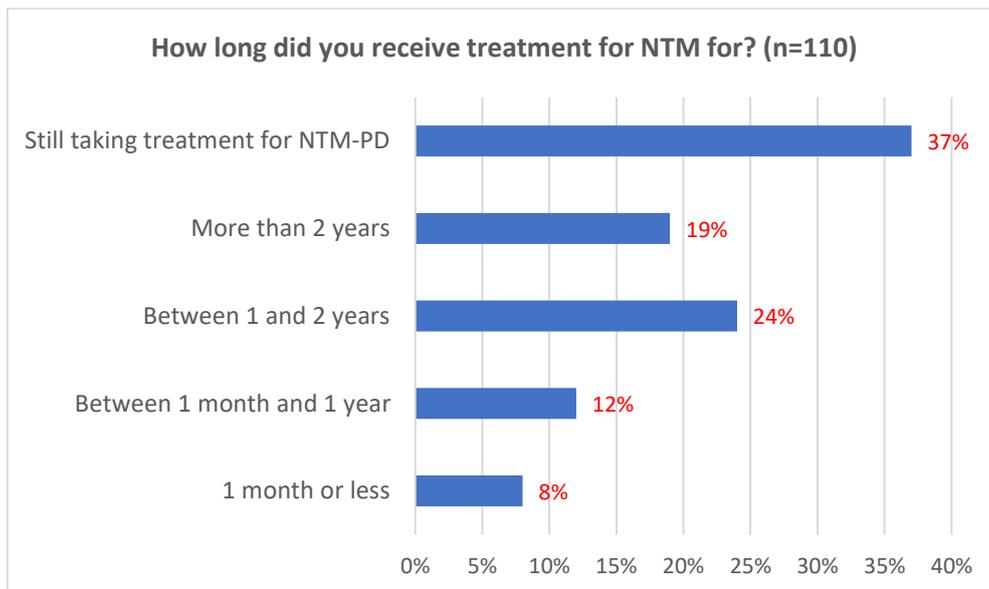
Other: 14% of respondents stated that 'Other species' were found and the following were specified: M. Fortuitum e m.chelonae; Mycobacterium chelonae; pseudomonas a; Streptococco; Fortuitim; Pseudomonas; Simiae; Fortuitum and Chelonae; Just told A Typical?; Mycobacterium simiae; Pseudomonas + aspergillus.

10. Respondents were asked if they were offered treatment for NTM-PD and 81% (n=118) stated 'Yes':

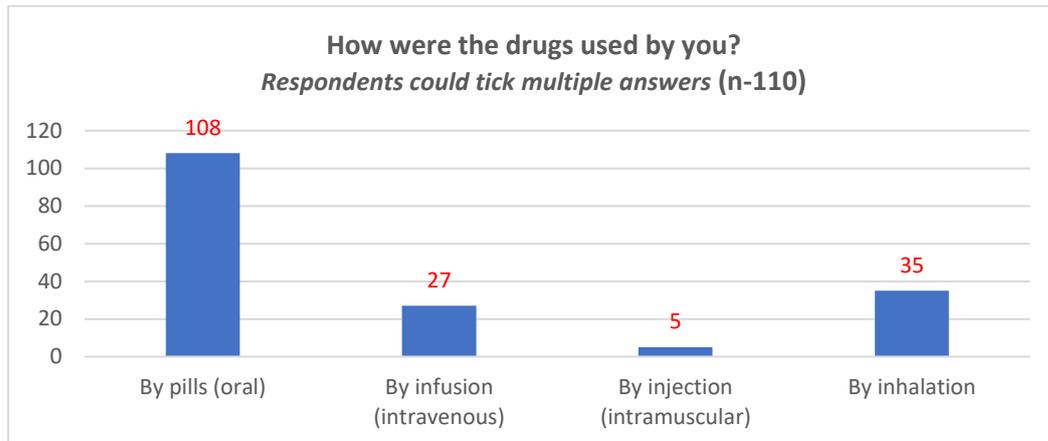


The following 3 questions were asked to those respondents who stated that they had been offered treatment:

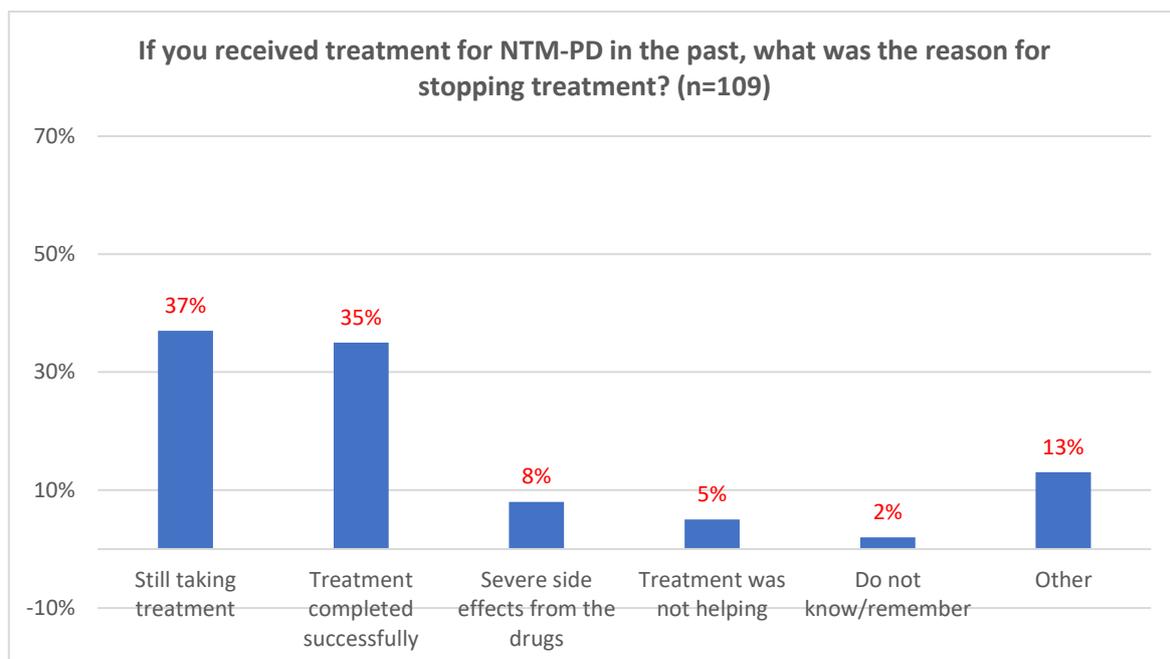
11. How long did you receive treatment for? 24% received it for between 1 and 2 years and 37% are still undergoing treatment:



12. Respondents were asked about the delivery of drugs used to treat their NTM-PD (respondents were able to tick all options that applied):



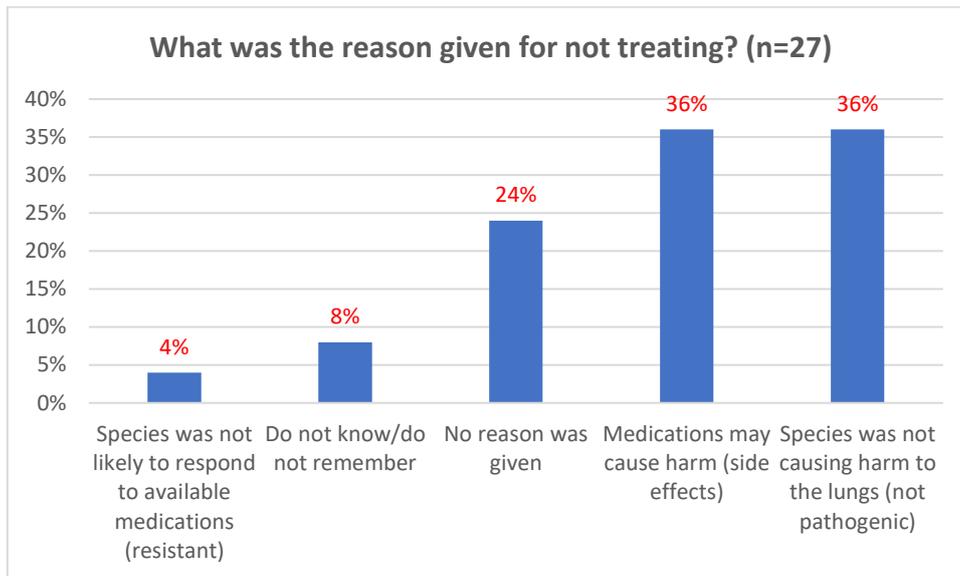
13. Respondents were asked about the reasons given for stopping their treatment and 35% stated the reason for stopping was because the treatment had been completed successfully while 37% were still undergoing treatment:



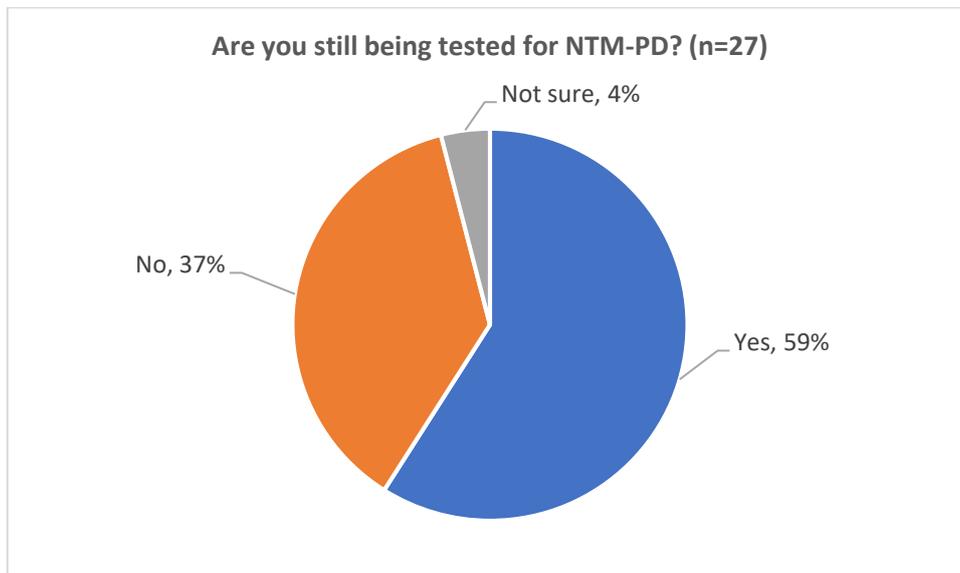
Additional reasons for stopping treatment were specified as:

- I chose not to have treatment but to watch and wait.
- Treated multiple times: some did not help, some could not tolerate, some successful.
- I was also diagnosed with granulomatosis polyangiitis within a month and my pulmonologist and infectious disease doctor felt most of my symptoms were coming from this and they needed to concentrate on treating that. Also, a bronchoscopy culture was negative for NTM while a sputum culture close to the same time was positive.
- Culture numbers were down, symptoms were less and to give my body a break from all the antibiotics and their side effects.

14. The 27 respondents (18%) who were not offered treatment gave the following reasons:

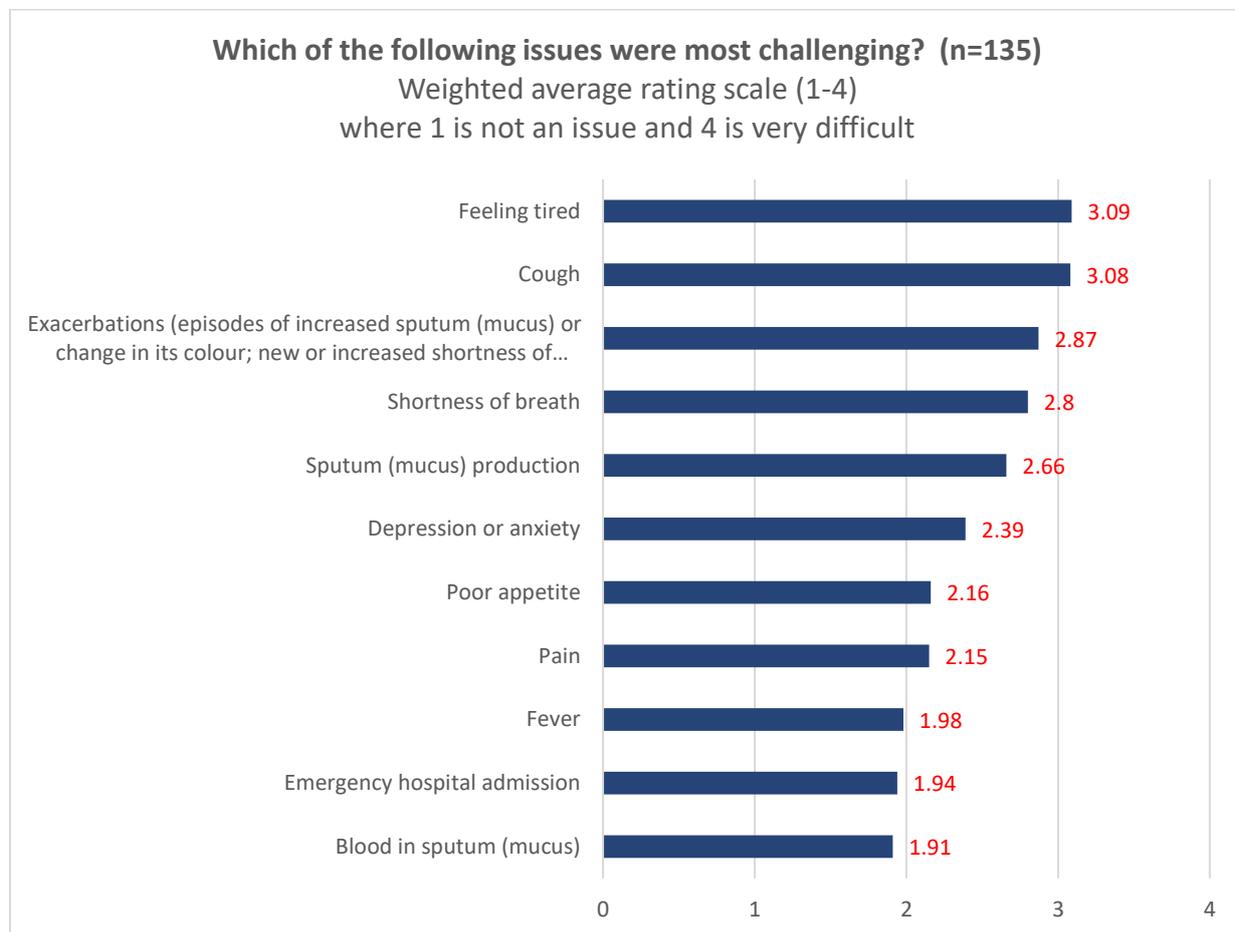


15. These respondents were also asked if they were still being tested for NTM-PD and 16 (59%) were still being tested; 10 people (37%) were no longer being tested and 1 person (4%) was not sure.



C. Challenging issues

16. The most challenging issues for those diagnosed with NTM-PD are shown in the chart below with 'Feeling tired' and 'Cough' identified as the top two most challenging issues. Note that only the bottom 3 issues were rated below the weighted average of '2'.



Some additional comments made about challenging issues faced are highlighted below:

a) Anxiety

- I feel very anxious about my respiratory condition becoming worse and then to have these bacteria and not treatment I feel quite scared..... this has severely impacted my mental health in a negative way.
- Anxiety due to not getting answers and tests sooner.
- Fear of facing further relapses (*Google translation of Italian*).
- Being told that the MAI is culturing more quickly, as this is worrying - it causes more distress. Also being told that treatment options are running out.

b) Side effects

- Night sweats; weight loss.
- Serious side effects from antibiotics; also heart, kidneys, tinnitus, nerve damage.
- Body reactions, including antibiotics such as: Autoimmune Disease "Subacute Cutaneous Lupus Erythematosus"; Skin redness, itching and fever; tinnitus, diarrhea; weight loss; nerve irritation and numbness (*Google translation from German*).
- Powerless, short of breath when moving (*Google translation from German*).

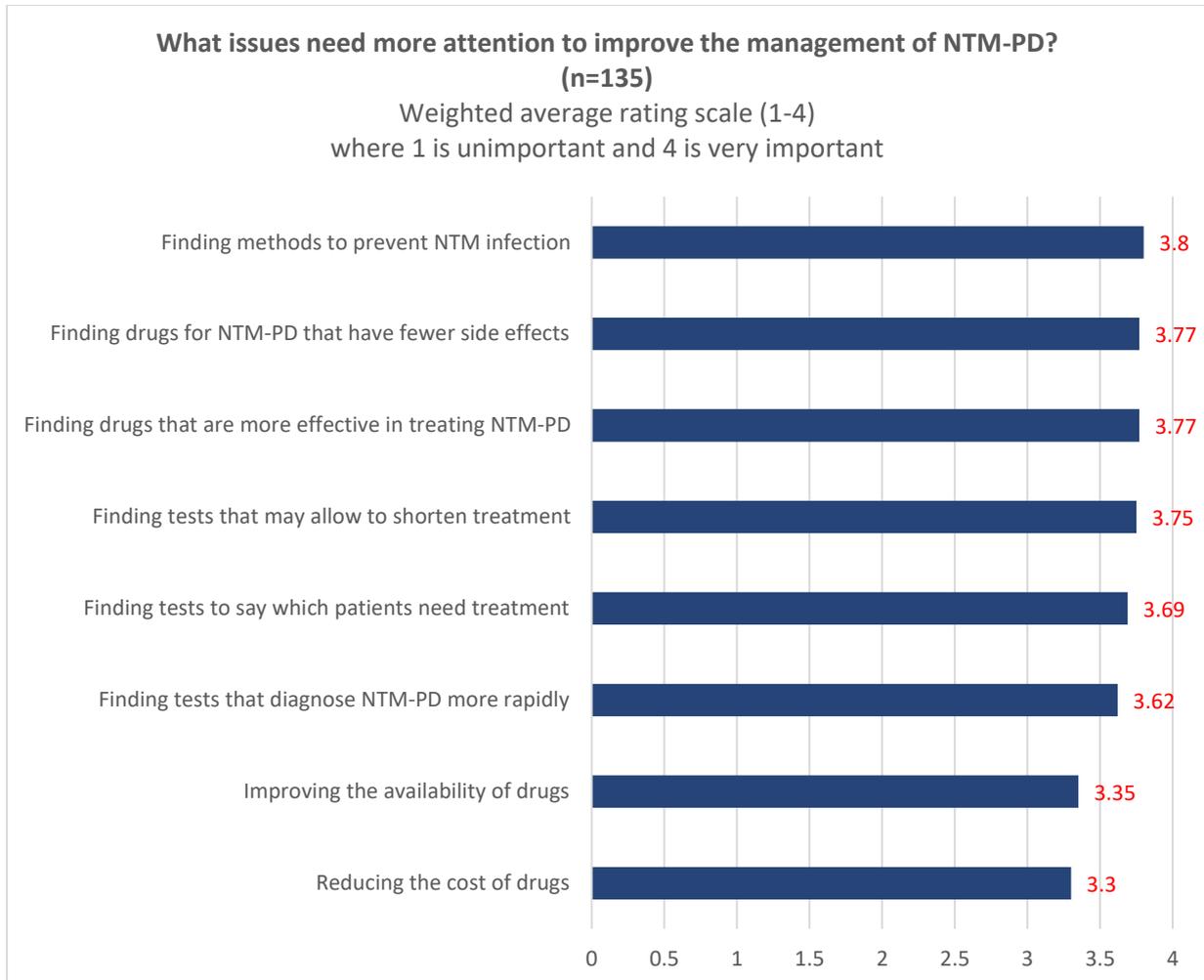
c) Treatment and care

- Urgent need for new, accessible, cost-effective treatments with minimal side effects.
- Excellent and rapid hospital diagnosis; good hospital follow-up but no community care support whatsoever.
- It is difficult for me to determine which disease is responsible for symptoms.
- Antibiotic treatment was difficult.

d) Impact on daily living/quality of life

- Not being able to work full time, needing oxygen at high altitudes, having to sell home and move to sea level.
- Weakness, isolation, inability to do things once enjoyed.
- The daily health regimen is so time consuming and has many parts that must be done in a certain order. There are pills, inhalers, nebulized medicines, respiratory vest, sinus rinse – and then all the dang cleaning of all the equipment.

17. The top 3 most important issues needing attention to improve the management of NTM-PD were identified as: Finding methods to prevent NTM infection; Finding drugs for NTM-PD that have fewer side effects; and Finding drugs that are more effective in treating NTM-PD. Note that the average weighting of all issues was between 3 and 4.



Additional issues needing attention that were included in the 'Other' comments box:

a) Raising awareness

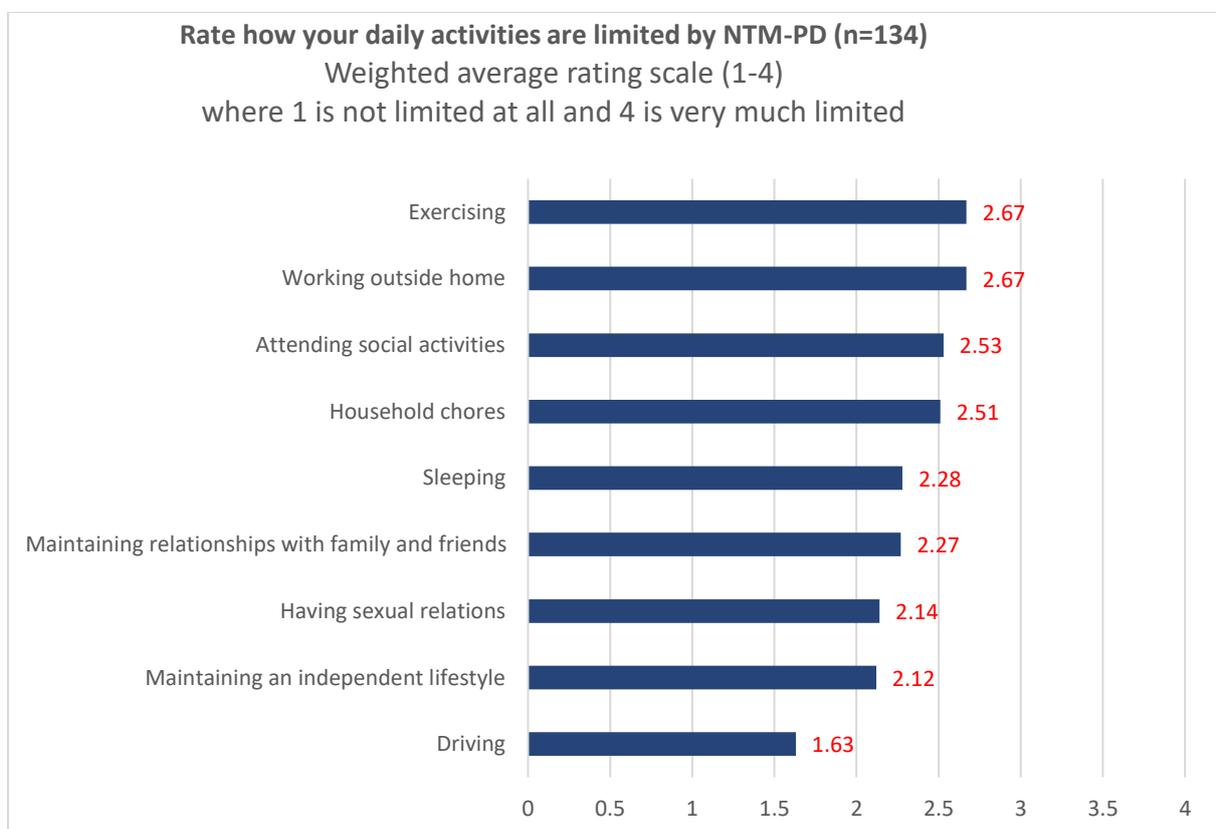
- Need to raise awareness of NTM amongst all healthcare professionals including GPs.
- Most doctors are not very careful, little informed, often underestimate symptoms.
- Raising public awareness and reducing stigma of the disease.
- Educate and arm patients with tools to prevent recurring infections.
- Helpful to know if the variety you have is -soil or water-based.

b) Treatment and research

- Develop an NTM vaccine to be given along with the TB vaccine for babies in high risk areas? Could vaccine be given to people with a weakened immune system too?
- Improving accuracy of culturing NTM / finding consistent medical opinion.

- Development of new drugs that would really be effective with very few side effects OR side effects that are not lasting.
 - More labs that are proficient at proper testing of sputum.
 - Avoid invasive investigations, such as bal bronchoscopy, which caused two pneumothoraxes to me (*Google translation from Italian*).
 - Learning about progression of the disease, about factors that might help define who can wait to be treated (I understand that in some cases people just never get worse), about lung conditions and environmental factors that could contribute to getting sick or having a recurrence after treatment.
 - Resistance research for alternative drug combinations with access for doctors:
 - o better networking of the researching countries
 - o fewer hurdles for tested medication from abroad
 - o more clinics should be involved
 - o Full state financial support for research in Germany to improve the profit behavior of the
 - o Contain pharmaceutical industry
- [Google translation from German]*
- I am asymptomatic, so I am not currently on the big 3 meds. However, the meds currently available leave much to be desired in terms of effectiveness and side effects. They are 20th century meds being used to treat a 21st century disease.

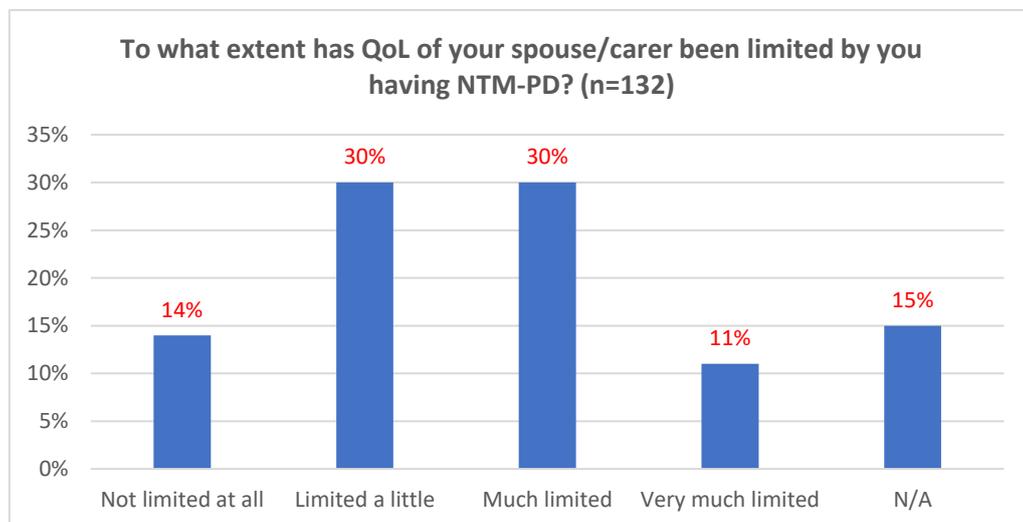
18. The top 3 daily activities most limited by NTM-PD were rated as: Exercising; Working outside the home; Attending social activities. See chart below for ratings of all activities:



Additional impact on daily activities that were included as comments in the 'Other' box:

- Dancing and hiking and any sustained physical activity... being among people in social situations because of fear of contracting infection... air travel limited due to amount of germs in plane and at airport.
- I am 'stable' at the moment and was a keen gardener. I'm not feeling very safe in the garden. I also used to go to the gym and use the indoor pool, steam room etc. but have stopped this. I feel that I would benefit from swimming but am too worried now.
- All of these are dependent upon how I feel each day.
- Constant coughing and sputum production prevent a normal lifestyle. Pain and tiredness from the coughing. Hearing loss from medication has destroyed my life.
- I had so many episodes of severe hemoptysis that I became afraid of leaving the house, for fear of having an episode in public.
- All the nebulisers and physio take so much time.
- No social life whatsoever as no one understands severe respiratory disease and extreme dyspnoea.

19. The survey asked how much the quality of life of the respondent's spouse/carer had been limited by NTM-PD with 60% rating this as being either 'limited a little' or 'much limited':

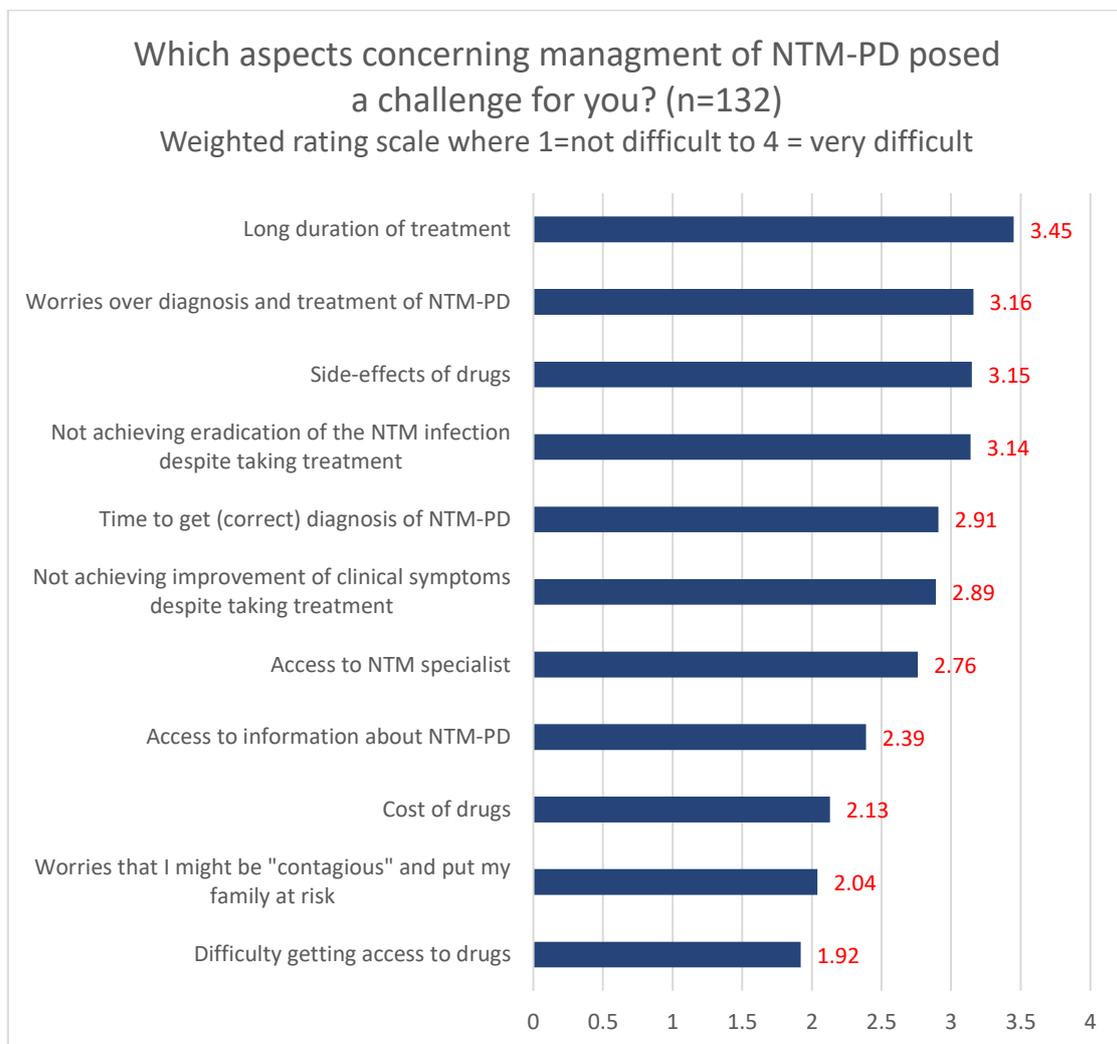


Additional comments made about impacts on quality of life of the spouse/carer:

- Tiredness and not being able to plan anything.
- My carer (husband) is elderly and quite frail in himself. My depression is hard for him and my inability to do household chores. Also, my reluctance to go out at times.
- Can no longer work.
- My husband/caregiver tries to be as normal as possible around me, but I know he is suffering emotionally... his desire to "fix" my health problems makes him frustrated.

- He has been very supportive and he was very reluctant to leave me alone and our whole life seemed to revolve around doctors' visits.
- He has to listen to me cough and clear my throat and cough up sputum all of the time. We can no longer go hiking. I can no longer help with yard work. Since I have soooo much cleaning of all of my respiratory equipment each day, he graciously does most of the dishes. Most of the time I cannot lay on my back to make love, since laying on my back causes coughing spells that can last for hours.
- He has gone from being my husband to more of a caregiving role. He does not complain, but it is not what he signed up for.
- Not able to travel or do sport with him. He knows I will die fairly soon.

20. See responses to which aspects concerning management of NTM-PD posed a challenge for you? The most difficult aspects were rated as: 'Long duration of treatment'; 'Worries over diagnosis and treatment of NTM-PD'; 'Side-effects of drugs' and 'Not achieving eradication of NTM infection despite taking treatment':

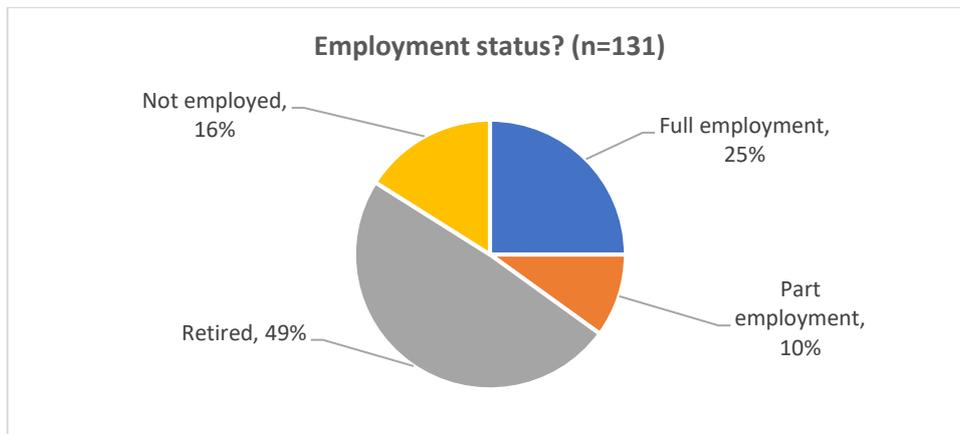


Other challenging aspects of management included in the 'Open' field box:

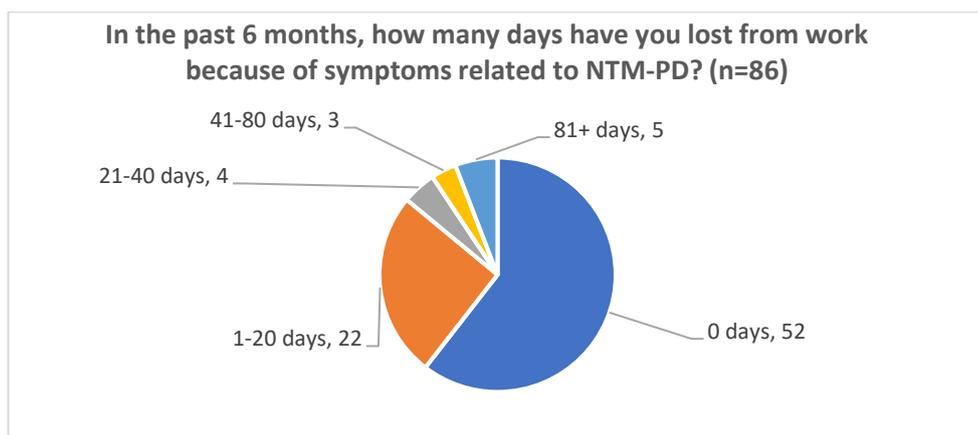
- The high probability of contracting the infection again.
- Knowing which bug is causing problems e.g. pseudomonas on top of NTM.
- Having a clear understanding of CT results e.g. cavities, nodules.
- It is very difficult to cover the costs through the health insurance companies of those not yet approved here (*Google translation from German*).
- Antibiotic combinations must be tested by the treating physicians themselves.
- Fatigue was horrible.
- Managing the yeast infections that come with all of the antibiotic use.
- The medications available don't work, destroy your body, cost a fortune, Not easy to find a Dr that is knowledgeable about this disease.
- It took an unusually long time for my hemoptysis episodes to subside. I kept landing in the ER and the ICU. I lost hope, and thought, for a while, that I should end treatment, and simply go home to die. I was not yet 50 years old.
- Difficulty getting drips and picc lines in.
- Reluctance of specialists to make any changes to treatment, despite it not working.

D. Impact of NTM-PD on work productivity

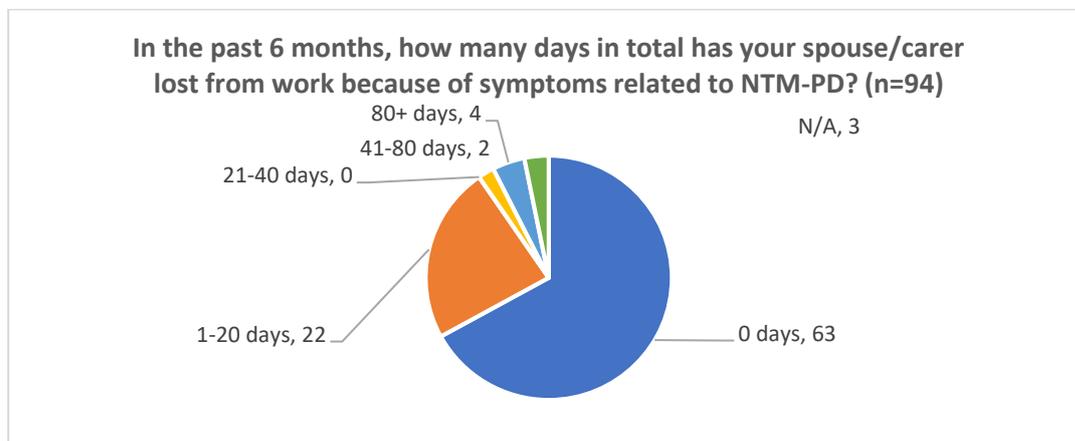
21. Respondents were asked about employment: 49% retired; 25% in full employment:



22. The majority (n=52) had lost 0 days at work during last 6 months with 22 respondents losing between 1-20 days:



23. The majority (n=63) of spouse/carer had lost 0 days with 22 stating between 1-20 days had been lost:



Additional important comments

24. Respondents were asked to add any other important comments about NTM-PD. These have been grouped into topic areas although there is some overlap:

a) Education/ raising awareness

Many comments were made about the need for more education of medical professionals about NTM. Some specific comments are below:

- The ignorance in the medical community that only immune compromised individuals or those with HIV have NTM needs to be improved. Not so much with infectious disease but the pulmonary community needs to be educated better. Otherwise healthy individuals such as myself wind up with this disease and are treated like HIV positive people until testing shows otherwise.
- More medics from all areas need to be made aware of NTM - and to recognise the symptoms to make diagnosis easier and quicker.
- My biggest concern is about lack of knowledge out there about this disease. I seem to get more info from the MAC forum on Facebook and from videos from Jewish Health in Denver than I do from my own doctor. Often it is conflicting.
- Doctors need to be VERY EDUCATED about disease/treatment or refer to specialist.
- Misinformation about what causes it.

b) Quality of life

- Makes me lead a much quieter, less active life. I've had to struggle with the reality of the situation.
- At my lowest, this illness took away my desire to live. I really just wanted to give up. And my medical team seemed to think that a life spent at home, waiting for the next episode of hemoptysis, so that I could go running to the ER, was an acceptable quality of life. It really is not, nor should it be.
- The fact that serious infections interfere with being able to arrange appointments e.g. not only medical things but hairdressers, ophthalmology, general appointments which allow us to function. Then there are very often the cancellations due to feeling very ill from this infection and it's difficult to get people to understand.
- Retiring from work early due to deterioration in respiratory health alongside having these infections affect your financial status.

- The lack of support and feeling ill constantly makes me feel I'm slowly dying and no one is there to care or befriend me that understands.
- I feel anxious that my life is slipping away and I'm not living how I hoped I would and grieve for the person I was.
- I'm desperate to be supported with information, medication and some type of counselling related to the condition maybe could be from lung foundation and online support where you can ring people.
- I only work very part-time on a self-employed basis, because fatigue from the various lung conditions won't allow me to go out to work in my registered profession.

c) Diagnosis / specialists

- We need faster diagnosis. I lost decades being on the wrong medications, 5 teaching hospitals missed it.
- It should be more readily considered as a diagnosis option by GPs. It took several years, and my persuasion, to get my doctor to refer me despite abnormal x-ray results spread over 2 years.
- I am a doctor and did think my TB had returned. People who have had a childhood TB infection need to know that this NTM is possible later in life.
- The availability of NTM-PD specialists is incredibly important. I was extremely lucky that following a hospital emergency admission, my respiratory consultant diagnosed m-Kansasii immediately, i.e. before sputum lab tests confirmed his diagnosis.
- Make use of non-invasive tests. To make the pathology recognized as a rare disease, or less, penalizing to help also for tax and social security purposes as well as for exemption from the payment of all the necessary drugs and tests (*Google translation from Italian*).
- I wish I had known more when first diagnosed. I thought I only had to take the drugs for a year and would be cured. I was told I was cured. A year later it was back, treated for another year, told I was better, then it returned worse than before. 16 years since diagnosis.
- I feel it's important to know about other comorbidities and/or cause of the infections.

d) Treatment and care

- Drugs to avoid reinfection (if prior treatment was successful).
- More information about avoiding reinfection would be good as there seems to be conflicting advice.
- Resources to help us to NOT get MAC again: air filters and water filters and other information to keep us safe from getting MAC again and again!
- A standard protocol in treating NTM's.
- This condition should be reportable so that we have data of the real number of people affected. The bigger the population, more pharmaceuticals will be motivated to invest for research for cure (not just treatment).
- I tested sputum positive for M Chelonae. I then underwent a bronchoscope which grew my usual bronchiectasis bacteria but was NTM negative. Since then I have no repeat testing. This caused cross-infection issues in my clinic and hospital. I think that specific clinics set up for NTM patients would be ideal from the point of sputum positivity, so that expert decisions regarding diagnosis, testing and treatment can be made. It seems that this is super-specialist even for bronchiectasis specialists.
- The state does not help me both psychologically and economically and with a job or a pension this depresses even more (*Google translation from Italian*).
- Lack of updated hospital centers on the specific treatment of bronchiectasis, long times to access specialist visits, lack of ticket exemption for this pathology (*Google translation from Italian*).
- Interested in phage therapy as it sounds like it has fewer side effects. Side effects are a constant concern besides being ill.
- Sputum tests are difficult...my mouth is so dry it's difficult to get spit up after a night of fasting.
- I have bronchiectasis as well and many of the symptoms I've ticked may well be due to that rather than NTM, I have no way of knowing.
- Not really knowing if how I felt was from the infection, medication or depression.

e) Research

- Find a drug that helps without many serious side effects and not so long ingestion. (*Google translation from German*).
- I feel lucky that I am being treated by a specialist in infections such as ntm, but more research needs to be done. I would welcome more advice on how I can help myself to avoid infection other than being pumped with antibiotics for years.
- Are there other drugs that can help that aren't so dangerous and last so long? What happens if I cannot tolerate the medications?? Is this a death sentence???
- My issue is that it takes 12 weeks to see if you are reinfected. I read there is a dna probe that is in research. Needs fast tracked. You need research on how we are getting it not more drugs.
- With the increasing number of patients being diagnosed, it is of utmost importance the development of new drugs that will be effective with minimal side effects.
- My concerns stemmed from fear of making things worse through showering, tap water and breathing in dust outside while on yard duty. (I'm a teacher).
- More research into small airway disease.
- Need to find a cure for this disease! The medications that are being used now are not targeted for this disease. All these meds have caused havoc on my body and yet have not destroyed the bacteria. Also need to find out why there is an increase of people getting this, and why more women than men?
- Difficult for me to have pulmonary rehabilitation in my hospital.
- Finding a specialist is very hard and I had to go private initially, and travel.