# RESEARCH

Insights from the European Nontuberculous mycobacterial pulmonary disease PAtient Disease Experience (ENPADE) survey– exploring disease burden and impact

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# Abstract

**Background** Nontuberculous mycobacterial pulmonary disease (NTM-PD) poses substantial diagnostic and management challenges, particularly among individuals with pre-existing lung conditions and/ or immunodeficiencies. NTM-PD can severely impair lung function and quality of life, potentially leading to both increased healthcare costs and mortality. There is a lack of comprehensive understanding of the disease burden and healthcare gaps from the patients' perspective. The European NTM-PD Patient Disease Experience (ENPADE) survey aimed to collect insights into these aspects.

**Methods** The survey aim was addressed by several methods. First, an online questionnaire was carried out from July 2021 to February 2022 across eight European countries for quantitative data collection. Additionally, semi-structured qualitative patient interviews were conducted with a subset of patients, eliciting their insights on the aspects surveyed. Descriptive statistics were used for quantitative analysis and interview outcomes were categorised along the online questionnaire for qualitative analysis.

**Results** A total of 543 patients participated in the survey and 23 patients were interviewed. Satisfaction with care received before and after diagnosis was scored, on average, moderate with 32% "highly satisfied" patients and 25% "highly dissatisfied" patients across the aspects surveyed. Dissatisfaction was expressed particularly regarding referral and access to expert care, and information received on their disease and its management. Patients reported high restrictions in daily life (49%), work (31%), and social activities (43%), often leading to substantial emotional distress, such as experiencing an increase in feeling depressed or anxious (82%). Interviews with patients highlighted a need for improved disease information, faster diagnosis, and enhanced physician-patient relationships.

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**Conclusions** The ENPADE survey outcomes revealed dissatisfaction among patients with care and restrictions in daily life, work, and social activities, often leading to emotional distress. These findings underscore the need for improved disease information, standardised care, and enhanced physician-patient relationships with appropriate support measures.

**Keywords** Nontuberculous mycobacterial pulmonary disease, NTM-PD, Online survey, Patient experience, Burden of disease

# Background

Nontuberculous mycobacterial lung disease (NTM-PD) is a growing health concern due to its complexity and the substantial challenges it poses in terms of diagnosis and management. These are particularly challenging in vulnerable populations like those with pre-existing chronic lung conditions and/ or immunodeficiencies [1–6]. Patients with NTM-PD may suffer from a range of non-specific symptoms beyond respiratory complaints that essentially impair overall quality of life [7]. The diversity of symptoms and the potential overlap with pre-existing symptoms related to an underlying lung disease may contribute to a delay in diagnosis of NTM-PD which may take years [8]. These difficulties may all lead to substantial healthcare expenses [8].

As reflected in the 2020 ATS/ERS/ESCMID/IDSA guidelines, NTM-PD management involves prolonged multidrug regimens, which are often complicated by drug adverse effects and subsequent suboptimal treatment adherence [9]. The challenges patients face, from prolonged treatment courses to the adverse effects of drugs, remain partially recognized. This was highlighted in a survey conducted by the European Multicenter Bronchiectasis Audit and Research Collaboration (EMBARC) and the European Lung Foundation (ELF), that stressed the urgent need for education and patient-centered care approaches to address the NTM-PD management and gaps in patient care [10].

Adding to the EMBARC-ELF survey, the present European NTM-PD Patient Disease Experience (ENPADE) survey was designed to further investigate the daily challenges in receiving care and impact on daily life, work, social activities, and emotional wellbeing that patients with NTM-PD experience.

# Materials and methods

# Survey design

The ENPADE survey was conducted from June 2021 to February 2022 across eight European countries (Austria, Belgium, France, Germany, Italy, the Netherlands, Spain, and the United Kingdom). The survey included a self-administered online questionnaire (Part A) and, for a subset of patients included in Part A, qualitative semistructured patient interviews (Part B).

Patients included in the survey were adults from the eight European countries diagnosed by a physician with NTM-PD (self-declared in the online questionnaire without external validation) who stated either receiving treatment (antibiotic therapy and/or other therapy) for NTM-PD or not receiving treatment but having NTM-PD symptoms (e.g. cough, fatigue, fever, loss of appetite, night sweats, weight loss).

Patients were recruited to the online questionnaire via a landing page using multiple channels (online advertisements, e.g. Google Ads; social media, e.g. Facebook; patient organisations associated with a higher risk for NTM-PD; healthcare waiting rooms, e.g. supportive pulmonology departments of clinics) in each country.

## Part A- quantitative online questionnaire

The questionnaire was developed iteratively by a core team of the authors, based on studies of previous NTM-PD surveys, considering FDA's Patient-Focused Drug Development (PFDD) Guidance 2, and with the input of two patient experts in a review process [1, 10, 11]. The questions focused on treated and/ or symptomatic NTM-PD patients (See additional file 1). It was initially designed in English and translated by a certified translation agency into Dutch, French, German, Italian, and Spanish. The 33 questions covered topics on patient characteristics, experiences with various aspects of NTM-PD care (care prior to diagnosis, at diagnosis and during treatment, information provided by healthcare professionals (HCPs), and experiences with drug therapy) and the impact of the disease on daily life activities, social life, and emotional wellbeing. We did not include a specific health-related quality of life questionnaire, but rather, focused on general questions on quality-of-life burden. Responses were anonymously collected via a browser-based online survey tool (SurveyMonkey) and analysed using descriptive statistics (mean, median, ranges, and standard deviation [SD]) in Microsoft Excel. If the data allowed, stratifications were performed for country-specific health care aspects and to investigate further research questions in more detail.

All responses were analysed for all questions that the participants replied to, regardless of whether they fully completed the questionnaire. Premature survey termination by participants resulted in a decrease over time in the number of responses per question.

#### Part B- qualitative semi-structured interviews

All patients who completed the online questionnaire were invited to participate in semi-structured interviews conducted in the same time period as Part A via virtual communication platforms (Microsoft Teams or Zoom). There were no further criteria applied to the selection of interviewees. Interviewers used open-ended discussion guides developed based on the online questionnaire to gain in-depth insights in the aspects of care and impact of disease that were addressed in Part A (See additional file 2). All participants were interviewed in their native language by patient engagement professionals of admedicum GmbH & Co KG (admedicum) experienced and trained in conducting interviews with patients. Interviews were subsequently summarised and translated into English by admedicum. Additional insights gathered from the in-depth interviews are highlighted in the appropriate section of Results, along with the results from the online questionnaire.

# Results

# **Patient characteristics**

Of 2325 patients who started the online questionnaire, a total of 543 NTM-PD patients from 8 European countries met the inclusion criteria and 49% (n = 268) completed the entire online questionnaire and were invited to participate in an interview (Table 1). The majority of participants in the online questionnaire were from the United Kingdom (*n* = 185, 34%), Germany (*n* = 111, 20%), and Italy (n = 87, 16%). A significant proportion of the patients were aged over 50 years (82%), most respondents were women (73%) and just over a third (37%) held higher education degrees. The most commonly reported comorbidities were chronic obstructive pulmonary disease (COPD) (27%), bronchiectasis (25%), asthma (12%), and impaired immunity (11%) (Table 2). Approximately onethird of the patients (31%) were diagnosed over 5 years ago and 28%, less than one year before the survey. Respiratory or infectious disease physicians in a local practice

Table 1	Online c	uestionnaire	completion	rates
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or hospital treated 61% of patients, while 16% saw an expert in a specialist centre for lung diseases, particularly in Belgium and Italy (33% and 22%, respectively). General practitioners managed 8% of patients, however, that proportion was notably higher in the United Kingdom and Germany (18% and 14%, respectively).

Data from 23 semi-structured qualitative interviews were obtained from patients across 7 countries (France = 7, United Kingdom = 6, Germany = 5, Spain = 2, Italy = 1, Austria = 1 and Belgium = 1) and provided qualitative insights into the topics included in the online questionnaire. The interviewees, predominantly female (87%), had a mean age of 61 years (range 33–72 years) and a similar demographic profile to the online questionnaire respondents.

# Therapy received and responsible physicians

Among the surveyed patients, 75% had received treatment (of whom around 80% reported still undergoing treatment at the time of the survey), 15% were monitored but not on treatment, and 10% were neither treated nor monitored by physicians, with half of these coming from the United Kingdom (Table 3).

Most treated patients (97%) reported having received any kind of drug treatment, 7% had undergone lung surgery, and 2% mentioned lung transplants. Airway clearance therapy and/or physiotherapy was reported by 38% of treated patients.

#### Patient satisfaction with aspects of NTM-PD care

Patient satisfaction– rated on a scale from 1 (very dissatisfied) to 10 (extremely satisfied)– revealed moderate satisfaction with an average rating of 5.9.

On a closer look, 26% of patients were highly dissatisfied with various aspects of care in the pre-diagnosis period and one-third (33%) were highly satisfied (See additional file 3 and additional file 4 for country specific results). The time it took to get referred to a specialist was the aspect patients were least satisfied with

Country	Qualification criteria (Q1-Q5)	Diagnosis (Q6-Q13)	Therapy (Q14-Q20)	NTM-PD Impact (Q21-Q30)	Final demographics/ comorbidity (Q31-Q33)
	% (n)	% (n)	% (n)	% (n)	% (n)
United Kingdom	100.0% (185)	44.9% (83)	38.9% (72)	35.1% (65)	35.1% (65)
Germany	100.0% (111)	69.4% (77)	65.8% (73)	64.9% (72)	64.9% (72)
Italy	100.0% (87)	73.6% (64)	66.7% (58)	62.1% (54)	60.9% (53)
France	100.0% (56)	75.0% (42)	64.3% (36)	60.7% (34)	58.9% (33)
Spain	100.0% (38)	55.3% (21)	36.8% (14)	31.6% (12)	28.9% (11)
Belgium	100.0% (33)	69.7% (23)	60.6% (20)	57.6% (19)	57.6% (19)
The Netherlands	100.0% (22)	59.1% (13)	50.0% (11)	50.0% (11)	50.0% (11)
Austria	100.0% (11)	45.5% (5)	36.4% (4)	36.4% (4)	36.4% (4)
Total	100.0% (543)	60.4% (328)	53.0% (288)	49.9% (271)	49.4% (268)

Q = question; % = percentage of the number of respondents who completed the Q1-Q5 questions

# **Table 2** Patient demographics and comorbidities

	United Kingdom	Germany	Italy	France	Spain	Belgium	Netherlands	Austria	Total
Gender % (n)									
Female	70.8% (46)	79.2% (57)	62.3% (33)	90.9% (30)	63.6% (7)	63.2% (12)	72.7% (8)	50.0% (2)	72.8% (195)
Male	26.2% (17)	15.3% (11)	32.1% (17)	9.1% (3)	9.1% (1)	36.8% (7)	18.2% (2)	50.0% (2)	22.4% (60)
Prefer not to answer	3.1% (2)	5.6% (4)	5.7% (3)	0.0% (0)	27.3% (3)	0.0% (0)	9.1% (1)	0.0% (0)	4.9% (13)
Education % (n)									
No schooling completed	4.6% (3)	4.2% (3)	0.0% (0)	0.0% (0)	9.1% (1)	0.0% (0)	0.0% (0)	0.0% (0)	2.6% (7)
Primary education (4–11 years)	3.1% (2)	5.6% (4)	0.0% (0)	3.0% (1)	9.1% (1)	0.0% (0)	9.1% (1)	0.0% (0)	3.4% (9)
Secondary education (11–16/18 years)	23.1% (15)	38.9% (28)	30.2% (16)	12.1% (4)	18.2% (2)	31.6% (6)	27.3% (3)	50.0% (2)	28.4% (76)
Further education (vocational qualification, 16–18 years)	29.2% (19)	20.8% (15)	24.5% (13)	18.2% (6)	36,4% (4)	21.1% (4)	9.1% (1)	25.0% (1)	23.5% (63)
Undergraduate higher education (e.g. BA, Bsc)	20.0% (13)	12.5% (9)	13.2% (7)	36.4% (12)	9.1% (1)	31.6% (6)	36.4% (4)	0.0% (0)	19.4% (52)
Postgraduate higher education (e.g. Masters, PhD)	13.8% (9)	12.5% (9)	30.2% (16)	27.3% (9)	18.2% (2)	5.3% (1)	9.1% (1)	25.0% (1)	17.9% (48)
Prefer not to answer	6.2% (4)	5.6% (4)	1.9% (1)	3.0% (1)	0.0% (0)	10.5% (2)	9.1% (1)	0.0% (0)	4.9% (13)
Comorbidities									
COPD	31.7% (39)	35.8% (44)	12.2% (15)	8.9% (11)	3.3% (4)	3.3% (4)	3.3% (4)	1.6% (2)	27.1% (123)
Bronchiectasis	16.8% (19)	15.0% (17)	35.4% (40)	8.8% (10)	6.2% (7)	10.6% (12)	6.2% (7)	0.9% (1)	24.9% (113)
Asthma	30.8% (16)	30.8% (16)	11.5% (6)	11.5% (6)	3.8% (2)	7.7% (4)	3.8% (2)	0.0% (0)	11.5% (52)
Impaired immune system*	18.4% (9)	32.7% (16)	26.5% (13)	6.1% (3)	6.1% (3)	8.2% (4)	2.0% (1)	0.0% (0)	10.8% (49)
Frequent pneumonia	11.4% (4)	37.1% (13)	17.1% (6)	8.6% (3)	5.7% (2)	5.7% (2)	14.3% (5)	0.0% (0)	7.7% (35)
Previous tuberculosis infection	25.0% (4)	18.8% (3)	31.3% (5)	0.0% (0)	6.3% (1)	18.8% (3)	0.0% (0)	0.0% (0)	3.5% (16)
Cystic Fibrosis (CF)	33.3% (4)	8.3% (1)	16.7% (2)	25.0% (3)	8.3% (1)	0.0% (0)	0.0% (0)	8.3% (1)	2.6% (12)
Whooping cough	33.3% (2)	0.0% (0)	33.3% (2)	33.3% (2)	0.0% (0)	0.0% (0)	0.0% (0)	0.0% (0)	1.3% (6)
Alpha 1 antitrypsin deficiency	20.0% (1)	60.0% (3)	20.0% (1)	0.0% (0)	0.0% (0)	0.0% (0)	0.0% (0)	0.0% (0)	1.1% (5)
Ciliary Dyskinesia	50.0% (1)	50.0% (1)	0.0% (0)	0.0% (0)	0.0% (0)	0.0% (0)	0.0% (0)	0.0% (0)	0.4% (2)
HIV-positive	0.0% (0)	0.0% (0)	0.0% (0)	0.0% (0)	0.0% (0)	0.0% (0)	0.0% (0)	0.0% (0)	0.0% (0)
Other**	14.6% (6)	26.8% (11)	19.5% (8)	22.0% (9)	9.8% (4)	7.3% (3)	0.0% (0)	0.0% (0)	9.0% (41)

This table presents two distinct types of data. The percentages and numbers for 'Gender' and "Education level' represent the distribution amount the patient population studied. The percentages and numbers for 'Comorbidities' represent reported events, not individual patients, as some patients may have multiple comorbidities. \*Patients who chose "impaired immune system" specified overall different forms of arthritis (n=5), forms of chronic inflammatory bowel disease (n=4) and sarcoidosis (n=2). \*\*Patients who chose "Other" specified overall forms of cancer (n=9), heart diseases (n=5), emphysema and other changes of pulmonary structure (n=9)

Tal	ble	23	0	verview	of	treatment/	′tł	herapy	received
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Country	Observed by physician without specific treatment/therapy	Neither treatment/therapy nor observation	Treatment/therapy received	Total % ( <i>n</i> )
	% ( <i>n</i> )	% ( <b>n</b> )	% ( <i>n</i> )	
United Kingdom	21.0% (16)	19.7% (15)	59.2% (45)	100.0% (76)
Germany	15.6% (12)	2.6% (2)	81.8% (63)	100.0% (77)
Italy	4.8% (3)	8.0% (5)	87.1% (54)	100.0% (62)
France	19.0% (8)	0.0% (0)	80.9% (34)	100.0% (42)
Spain	25.0% (5)	5.0% (1)	70.0% (14)	100.0% (20)
Belgium	4.3% (1)	17.4% (4)	78.3% (18)	100.0% (23)
the Netherlands	33.3% (4)	16.7% (2)	50.0% (6)	100.0% (12)
Austria	0.0% (0)	20.0% (1)	80.0% (4)	100.0% (5)
Total	15.4% (49)	9.5% (30)	75.1% (238)	100.0% (317)

Total population = 468 patients; Question did not specify which treatments/therapies are applicable

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Categories	Aspects	Ratir	ng 1-3	Rating 8-10
Care (pre-diagnosis)	Travel time needed to visit the expert physician specialised in treating NTM lung disease		20%	35%
Care (pre-diagnosis)	How long it took to get referred to the expert physician in treating NTM lung disease		30%	30%
Care (pre-diagnosis)	Effort needed to get referred to a specialist/specialized centre		27%	32%
Care (pre-diagnosis)	Finding an expert physician specialised in treating NTM lung disease		26%	31%
Care (pre-diagnosis)	Overall care received from first symptoms until the moment of diagnosis of my NTM lung disease		25%	39%
Care (during diagnosis/treatment)	Available time to discuss my diagnosis and treatment / therapy options		27%	35%
Care (during diagnosis/treatment)	Having to (partially) pay for treatments and / or therapies		17%	31%
Care (during diagnosis/treatment)	Clarity on the treatment / therapy goal		19%	49%
Care (during diagnosis/treatment)	Clarity on which physician is responsible for my NTM lung disease treatment / therapy		21%	51%
Care (during diagnosis/treatment)	Overall care I received while taking drug treatment(s) and / or receiving therapies for my NTM lung disease		22%	38%
Care (during diagnosis/treatment)	Support I received from physicians and other medical personnel so I could take care of my NTM lung disease even at difficult times during my daily life		25%	33%
Care (during diagnosis/treatment)	Consideration of my treatment preferences by my treating physician		20%	32%
Care (during diagnosis/treatment)	How long it took from first symptoms of NTM lung disease to the moment of diagnosis		27%	30%
Care (during diagnosis/treatment)	Level of confidence in physicians and other medical personnel with regard to diagnosing my NTM lung disease		20%	44%
Care (during diagnosis/treatment)	Burden of undergoing the diagnostic test procedures		19%	34%
Information	Information on NTM lung disease in general		31%	29%
Information	Information about my microbiological test results		27%	31%
Information	Information about treatment / therapy options to think about		28%	33%
Information	Information I received on possible side-effects of my treatment(s) and / or therapies		37%	29%
Information	Information I received on how my NTM lung disease is evolving over time		35%	35%
Information	Information on what I can do to support my NTM lung disease treatment / therapy		31%	31%
Drug therapy	Intravenous administration of drugs		13%	11%
Drug therapy	Administration of drugs through inhalation		18%	22%
Drug therapy	Tolerability / side effects of medications		28%	30%
Drug therapy	Number of drugs taken		35%	20%
Drug therapy	Perceived benefit of the treatment(s) / therapy or therapies (e.g. generally feeling better)		27%	29%
Drug therapy	Duration of treatment(s) / therapy or therapies		25%	28%
Drug therapy	Having to stay in the hospital to receive my medication(s)		10%	20%
	Average		25%	32%

Fig. 1 Overview of satisfaction ratings on different aspects of care (scale 1 ('extremely dissatisfied') to 10 ('extremely satisfied')). Total population differs between satisfaction aspects and can be found in the Additional file 3

(Fig. 1). Patients from the United Kingdom in particular reported higher dissatisfaction regarding the time and effort needed to get referred to a specialist compared to the overall sample, with 42% and 41%, respectively, rating these aspects very low (range 1–3).

Care during diagnosis and treatment, i.e. satisfaction relating to aspects such as clarity on treatment goals, and clarity on which physician is responsible for the patient's NTM-PD therapy, met higher satisfaction ratings compared to care in the pre-diagnosis period (Fig. 1). Still, aspects such as time available to discuss the patient's diagnosis and treatment options or time from first symptoms to the moment of diagnosis were evaluated as very low (range 1–3) on the satisfaction scale by 27% of patients.

Specifically, related to antimicrobial drug therapy, respondents expressed their dissatisfaction with the number of medications they needed to take, and tolerability issues associated with drug therapy (35% and 28% as highly dissatisfied, respectively) (See additional file 3 and additional file 4 for country specific results).

Finally, the aspect of care least meeting the expectations of patients related to the information patients received. Almost one in three patients (31%) expressed high dissatisfaction, particularly with the information received on possible side effects of their therapy (37%) and the information on how their NTM-PD is evolving over time (35%). Notably, 40% of German patients were highly dissatisfied with the information they received overall compared to 21% of Italian patients (See additional file 3 and additional file 4 for country specific results).

Individual interviews confirmed findings from the online questionnaire. Many of the 23 interviewees emphasized that their NTM-PD diagnosis was either an incidental finding or required consulting various physicians. Patients also expressed a desire for their concerns to be taken more seriously by their physicians. Most interviewees proactively sought information themselves, primarily using the Internet. A minority of interviewees (only two of the 23 interviewed patients) received supportive materials from their physicians about NTM-PD.

## **Burden of disease**

#### Daily impact

On a scale from 1 (not restrictive at all) to 10 (extremely restrictive), patients gave an average rating of 7 to the impact of NTM-PD on their daily life activities, with nearly half of the patients (49%) reporting serious restrictions (rating 8 to 10) (Fig. 2a).

#### Social life impact

On the same scale, patients with NTM-PD rated the impact on their social life at an average of 6.6. 43% of the surveyed patients reporting severe limitations (rating 8 to 10), reflecting a considerable impact on their social lives (Fig. 2a). Additionally, activities like maintaining friendships (average 5.4) and participating in social events (average 6.2) were affected, especially the latter,



**Fig. 2** Burden of NTM-PD on patients. (a) Impact of NTM-PD on daily life, social life, and family members, rated from 1 "not restrictive/impactful at all" to 10 "extremely restrictive/impactful". (b) Detailed view of social aspects affected by NTM-PD, with scores from 1 "not restrictive at all" to 10 "extremely restrictive". (c) Impact of NTM-PD on work, scored from – 10 "extremely negative" to 10 "extremely positive". (d) Overall emotional impact of NTM-PD, scored from – 10 "extremely negative" to 10 "extremely negative" to 10 "extremely negative" to 10 specific emotional states and changes in social behaviour. Participation in this question was optional

with 41% feeling highly limited in this activity (rating 8 to 10) (Fig. 2b). Interview responses echoed these findings, revealing that NTM-PD often disrupts physical, relational, and social engagement.

#### Work impact

At the time of NTM-PD diagnosis, approximately half the patients (54%) were employed. NTM-PD had a negative effect on their work, of an average rate of -4.1 on a scale from – 10 (extremely negative) to 10 (extremely positive). Almost a third (31%) reported severely negative impact (rating – 10 to -8) (Fig. 2c). Interviews provided further

insights, such as reducing job demands due to fatigue, or hiding the condition at work to avoid prejudice.

# Emotional impact on patients and their family members involvement

Patients rated the impact of NTM-PD on their emotional well-being on a scale from -10 (extremely negative) to 10 (extremely positive), with an average of -3.5 (Fig. 2d). Most patients (75%) reported a negative impact (rating – 10 to -1), with 11% giving the lowest possible score of -10 (Fig. 2d). Most patients reported experiencing increased depression or anxiety (82%) and nervousness (68%), while self-confidence, body image, and the willingness to perform activities were reportedly decreased due to NTM-PD (Fig. 2e). Interviews confirmed the emotional impact of NTM-PD, highlighting added stressors like disease concealment and its unpredictable nature. Additionally, the emotional impact on family members, on a scale from 1 (no negative impact at all) to 10 (extremely high negative impact), was moderate, with an average score of 4.1 (Fig. 2a). However, 19% reported severe impacts (rating 8 to 10), underscoring substantial distress in some families.

# Support services used to alleviate the burden of disease

Patients used various supportive services: 23% engaged in pulmonary rehabilitation, 17% sought nutritional support, and others used psychological support, smoking cessation support, and patient support groups (11%, 13%, and 5%, respectively). Interviews highlighted that, while some patients initiated psychological support themselves, many reported it was not offered, and attitudes towards accepting such support varied. On an emotional level, partners and other family members were the primary sources of support for 52% and 47% of patients, respectively.

### Discussion

Despite established treatment guidelines for NTM-PD, treatment practices, including the decision to treat and the treatment protocol, vary geographically, and adherence to treatment guidelines is not uniform [12-17]. Moreover, the current complex management, which requires multiple drugs often taken over 18 months and is associated with frequent adverse events, does not always achieve the goals of curing the infection and improving symptoms [8, 15, 18]. This survey of 543 NTM-PD patients, the largest research of this kind conducted in this disease area to date, reveals NTM-PD's effects on daily life and identifies potential areas for improvement in care from a patient perspective. About one-third of patients reported dissatisfaction with time from first NTM-PD symptoms to diagnosis. This finding reflects challenges in receiving diagnosis which may be due to limited disease awareness (by physicians and/or patients) and NTM-PD specific knowledge. As NTM-PD symptoms frequently overlap with those of other chronic respiratory conditions, timely diagnosis can be challenging. Recommendations regarding testing for NTM-PD in patients-at-risk (for example, those with pre-existing pulmonary conditions such as bronchiectasis) can contribute to reducing diagnostic delays [8, 19]. Low patient satisfaction with referrals and diagnostic timelines suggests that this is a key area for improvement. Wagner et al.. reported awareness gaps among physicians regarding NTM-PD, advocating for the establishment of clear referral pathways to specialist care for timely and accurate diagnosis [20]. Moreover, van Ingen et al. reported that the country of practice was influential on the decision to treat people with NTM-PD [12]. Overall, patients called for improved diagnostics, treatment options, and physician-patient relationships. Although patients' experiences with care were diverse, a notable proportion of patients was highly dissatisfied with the care they received, often related to the health care system organisation. The demand for standardised and expert-guided care is clear.

In this survey, patients expressed high levels of dissatisfaction with the information provided by their physicians, with only 5.3% feeling well-informed. This underscores the crucial need for educational initiatives to enhance patients' understanding. Given the rarity of NTM-PD, awareness and expertise varies across healthcare levels particularly on treatment regimens and potential side-effects, making dedicated centres for the care of NTM-PD highly desirable as this may considerably enhance disease management and improve patients' satisfaction and outcome. Educational background disparities further highlight the importance of accessible, patient-friendly resources that could be understood and useful, despite educational attainment. Providing reliable sources of information such as a comprehensive NTM-PD care website, patient-friendly brochures, and management tools available in local language would be highly beneficial. Patients should be closely involved in its development and these efforts should be accompanied with peer-to-peer support from patient advocacy groups. However, this is countered by the fact that today there are not many NTM-PD related patient advocacy groups in Europe and the establishment of such groups would first have to be supported.

The impact of NTM-PD on patients' daily lives, work, and social life cannot be overstated, often leading to substantial emotional distress, with many lacking support services to alleviate the disease burden. A comprehensive care model that spans beyond clinical treatment to address social, work-related, and psychological needs is necessary. A trans-sectorial, multidisciplinary approach, with regional expert centres cooperating closely, is important for successful disease management [1]. Based on the results of this patient survey, the authors suggest that healthcare services adopt a holistic care model, integrating patient's lifestyle, employment needs, and social support into the treatment plan. This change may include workplace adjustments, NTM-PD education for employers, and assistance navigating disability benefits. Regular mental health screenings and psychological support for patients and families are also suggested as essential services to increase patient satisfaction, and as a way to ensure that healthcare plans are tailored to individual preferences and evolving needs. Recently launched "Standards of care for people living with NTM disease in the UK," developed by the NTM Network UK is a good example of defining the minimal level of care that people with NTM-PD should expect to receive and clearly state that the psychological well-being of patients must be considered alongside physical parameters [21].

With 543 participants, the survey engaged a broad group of patients with NTM-PD, marking it as an essential contribution to patient insight on this disease. It is also important to consider certain limitations to fully understand the context of the findings. First, less than half of the initial respondents completed the questionnaire and completions are not proportional to the population of the countries involved, which might affect the representativeness of the results. Second, self-reported diagnosis of NTM-PD by patients was not externally validated through medical records. Third, the over-representation of female respondents (87% in this survey vs. approx. 50% in other population-based studies in Europe [22-24]) could reflect gender bias, as it has previously demonstrated that women are more respondent than men in participating in surveys. Fourth, online recruitment may not fully represent the real-world NTM-PD patient population. Finally, the virtual format of interviews may have limited the depth of responses, and a selection bias may have occurred due to a limited number of online respondents taking part in the in-depth interviews.

# Conclusion

In conclusion, this survey, the largest of its kind with 543 participants, revealed dissatisfaction among NTM-PD patients with care and restrictions in daily life, work, and social activities, often leading to emotional distress. These findings underscore the need for improved disease information, standardised care, and enhanced physicianpatient relationships with support measures.

#### Abbreviations

NTM-PD	Nontuberculous Mycobacterial Pulmonary Disease
COPD	Chronic Obstructive Pulmonary Disease
ATS	American Thoracic Society

ociety	noracic	American	ATS
ociety	noracic	American	ATS

ERS	European Respiratory Society
ESCMID	European Society of Clinical Microbiology and Infectious Diseases
IDSA	Infectious Diseases Society of America
ENPADE	European NTM-PD Patient Disease Experience
PFDD	Patient-Focused Drug Development
FDA	Food and Drug Administration
SD	Standard Deviation
HCP	Healthcare Professional

#### Supplementary Information

The online version contains supplementary material available at https://doi.or q/10.1186/s12890-025-03553-9

Supplementary Material 1: Additional file 1 Online guestionnaire in PDF format

Supplementary Material 2: Additional file 2 Interview guide for gualitative in-depth patient interviews in PDF format

Supplementary Material 3: Additional file 3 Excel file with data about satisfaction rating distribution for major countries

Supplementary Material 4: Additional file 4 Excel file with number of patients, mean and standard deviations of satisfaction aspects per country

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#### Author contributions

Survey conception and design: MO, RvL, CH, RL, AR; Data collection: CH, RL, MO, RvL, AR; Data analysis: CH, RL, MO, RvL, AR, MS, NL; Data interpretation: MS, NL, MO, RvL, CH, RL, AR; Manuscript writing and preparation: MS, NL, CH, MO, RL, RvL; Review and approval of final manuscript: all authors

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#### Data availability

The datasets generated and/or analysed during the current survey are not entirely publicly available due to the data privacy statement agreed upon with participants only sharing aggregated data. On reasonable request, the availability of datasets can be requested from the corresponding author.

#### Declarations

# Ethics approval and consent to participate

All participants consented to participate before beginning the online survey and prior to the interviews. Given the objectives, the intended use of the collected data, and the methodologies employed, this non-interventional research does not fall under any legislation requiring Institutional Review Board (IRB) approval in Austria, Belgium, France, Germany, Italy, the Netherlands, Spain, or the United Kingdom. In the extend applicable to its non-interventional nature, this research was conducted in accordance with the ethical principles of the Declaration of Helsinki. To comply with the general standards required for scientific publications, the survey obtained ethics approval from the French Institutional Review Board, Comité de Protection des Personnes, Ile de France IV, Hôpital Saint-Louis (N°IRB 00003835) under the registration number 2021-A01712-39 and methodological approval by the French Data Privacy Agency, Commission Nationale Informatique & Libertés under the reference number 2222822 v 0.

#### Consent for publication

Part A, the online questionnaire, only contains anonymous data and consent for publication was not applicable. Participants in Part B, the interviews, provided their consent for publication of pseudonymised data before participation in an interview.

#### **Competing interests**

MS received research grants from Novartis, Trudell pharma, GSK, and the Tel Aviv League for lung diseases; travel grants- Novartis, Actelion, Boeringer Ingelheim, GSK, Rafa. Speaker's fees- Astra Zeneca, Boeringer Ingelheim, GSK, Insmed, Teva, Novartis, Kamada, Sanofi. Advisory fees (incl. steering committee membership)- GSK, Boeringer Ingelheim, Kamada, Syncrony medical, Zambon, Vertex pharmaceuticals. DSMB Participation- Bonus therapeutics, Israel. RvL and MO are employees of Insmed, the company that funded the survey. CH, RL and AR are employees of admedicum, which received funding from Insmed to conduct the research. NL received consultancy fees from Insmed, GSK, AN2 Therapeutics, speaker's fees from GSK and Insmed, and a travel grant from Pfizer. All fees were provided to her institution, not to the author personally.

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