NTM Lung Disease: The Patient’s Perspective

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NTM Info & Research
Disclosures

No relevant financial disclosures to report

I will be discussing off-label uses of medications
Objectives

• Review tools and resources available to providers, patients, and caregivers to help educate patients about their illness and treatments

• Discuss patient experiences related to illness, treatments, and outcomes

• Discuss the role of new regulatory initiatives in future research, clinical practice, and therapeutic development
Co-founders: Patient & caregiver

The pamphlet: A resource for patients

The website: www.ntminfo.org
Online Forum for Patients

360Social
Resources for Patients

Local Support Groups

Research Pipeline Page
Bronchiectasis & NTM Research Registry
Additional Resources for Patients & Providers

Or give us a call:

305-667-6461
ext. 26 or ext. 32
Live Events
NTM & Bronchiectasis Conference

- CME-accredited Physician/Patient Conference (AMA, ANCC, ACPE, AARC, IPCE)
- May 14-15, 2020, Philadelphia, PA
- May 14: Patient sessions; May 15: clinical & scientific sessions
Patient-Centered Research Priorities for Pulmonary Nontuberculous Mycobacteria (NTM) Infection
An NTM Research Consortium Workshop Report

Emily Hennes,1 Timothy Alpers,2 Alan Barlow,3 Charles L. Duke,4 David Griffin,5 Philip Leiman,6 Amy Laframboise,7 Karen P. H. O’Dowd,8 Bobbi Pritt,9 Alessandra L. Radford,9 William Stash,10 John W. Watts,11 Kevin,12 Wendt13, and the NTMRC Patient Advisory Committee

Abstract
Nontuberculous mycobacteria (NTM) cause an increasingly important chronic and debilitating lung disease in adults. Diagnosis is often delayed, although awareness among clinicians and patients is increasing. Effective treatment options are limited, and outcomes vary according to the strain and severity of infection, making it important to develop new approaches to treatment. NTM Research Consensus (NTMRC) 2017. The NTMRC recognized the importance of these issues in developing research priorities for NTM. In November 2017, the NTMRC held a workshop to develop patient-centered research priorities for NTM lung disease. The workshop consisted of individuals who engaged in discussions to prioritize research opportunities for the future. The workshop focuses on improving diagnostic and treatment options and improving quality of life outcomes for individuals affected by NTM lung disease. The workshop participants agreed that two complementary objectives are necessary to ensure that the research priorities for the future are effective. The primary objective of the workshop was to develop research priorities for the future that are meaningful and relevant to patients and healthcare providers. The workshop agenda was designed to address the following key questions:
1. What are the most important research priorities for the future?
2. How can we ensure that research priorities are meaningful and relevant to patients and healthcare providers?
3. How can we ensure that research priorities are effective and can be implemented in practice?
4. How can we ensure that research priorities are sustainable and can be sustained over time?
5. How can we ensure that research priorities are funded and supported by external organizations?
6. How can we ensure that research priorities are communicated to patients and healthcare providers?

Table 1. Nontuberculous mycobacterial lung disease: patient-centered research priorities by topic area

<table>
<thead>
<tr>
<th>Topic</th>
<th>Priority</th>
<th>Potential Specific Questions and Next Steps</th>
</tr>
</thead>
<tbody>
<tr>
<td>Diagnosis</td>
<td>1. Strengthen the role of patients in preventing NTM infection or re-infection</td>
<td>Evaluate whether aspiration increases the risk of NTM infection or re-infection</td>
</tr>
<tr>
<td></td>
<td>2. Limit the risk of patient-to-patient transmission of NTM infection in cystic fibrosis clinics</td>
<td>Estimate the risk of person-to-person or indirect transmission in CF clinics</td>
</tr>
<tr>
<td></td>
<td>3. Improve the timeliness of diagnosis and develop protocols for rapid species identification and susceptibility testing</td>
<td>Comparative effectiveness of and expanded information control procedures for NTM infection</td>
</tr>
<tr>
<td></td>
<td>4. Develop a screening algorithm for patients at risk for pulmonary NTM disease</td>
<td>Validate molecular diagnostic techniques being developed by National Jewish Healthcare</td>
</tr>
<tr>
<td></td>
<td>5. Develop protocols for chemo-prophylaxis</td>
<td>Predictors of meeting ATS disease criteria at diagnosis</td>
</tr>
<tr>
<td></td>
<td>6. Reduce the impact on patients of anxiety and depression</td>
<td>Identity techniques that improve NTM's public health impact</td>
</tr>
<tr>
<td></td>
<td>7. Develop an NTM-specific Health-Related Quality of Life tool</td>
<td>Validate new symptom monitoring tools</td>
</tr>
<tr>
<td></td>
<td>8. Promote quality-of-life measures for assessing the effectiveness of treatment</td>
<td>Validate correlation between NTM Symptom Module and clinical outcomes</td>
</tr>
<tr>
<td></td>
<td>9. Reduce the burden of antibiotic treatment for NTM disease</td>
<td>Develop and evaluate alternative delivery systems for inhaled antibiotics</td>
</tr>
<tr>
<td></td>
<td>10. Develop and test the efficacy of non-pharmacologic therapies and holistic medicine approaches</td>
<td>Establish and maintain a network of experts in NTM clinical and academic settings</td>
</tr>
<tr>
<td></td>
<td>11. Improve understanding of what needs or benefits from therapy</td>
<td>Develop a composite index of disease activity or severity</td>
</tr>
<tr>
<td></td>
<td>12. Assess the composite measure of disease activity or severity</td>
<td>Identify biomarkers associated with disease risk, prognosis, and treatment response</td>
</tr>
<tr>
<td></td>
<td>13. Identify and validate biomarkers associated with disease risk, prognosis, and treatment response</td>
<td>Identify biomarkers associated with disease risk, prognosis, and treatment response</td>
</tr>
</tbody>
</table>

Definition of abbreviations: ATS = American Thoracic Society; CF= cystic fibrosis; NTM = nontuberculous mycobacteria.

*From Reference 32.*
Diagnosis: A Life Altering Moment

- What is it?
- Am I contagious?
- How will my life change?
- Will my family help me?
- Will my friends want to be around me?
- Am I getting the correct treatment?
- How long will I be on treatment?
- What are the side effects?
- Am I going to die from this?
Improved Quality of Life

NTM is **not** a passive disease.

*But some patients are more active decision makers/ participants in their own care than others.*

*In addition to taking medicine, your patient must commit to:*

- Airway clearance
- Nutrition
- Exercise and fitness
- Proper rest
- Psychological support mechanisms
Trust Yourself, Trust Your Patient

Listen to:
- Your patient
- Your patient’s caregiver
- Your patient’s other healthcare providers - work as a team

Treating patients with pulmonary NTM disease is a commitment on working with them. You must ask them to commit to their health as well and make them an active participant in their own care.
How Depression and Chronic Disease are Linked

Depressive symptoms are common in patients with COPD and those with severe COPD have a 2.5 times greater risk of developing depression.¹

Limitations on activity imposed by chronic medical illness may lead to gradual withdrawal from rewarding activities.²

Major depression is associated with increased medical utilization and costs, higher symptom burden, increased functional impairment³ and other decrements in quality of life, and increased rates of mortality.⁴

Major depression is associated with 50% to 100% higher costs in medical patients even after controlling for associated medical illness.⁵,⁶

4) Katon WJ. The depressed patient with comorbid illness. Program and abstracts of the 154th Annual Meeting of the American Psychiatric Association; May 5-10, 2001; New Orleans, LA. Industry Symposium, Part 2, 43B.
Look and Listen

• Is your patient feeling OK?
  • DEPRESSION
  • Plays a big role in chronic illness.
  • How does your patient LOOK?
  • Ask your patient how they’re FEELING.
• LISTEN to their answers for signs of depression.
What To Look For

- Sadness
- Anxiety/Fear
- Social isolation
- Anger
- Sleep disruption (too much or too little)
- Feeling of “not wanting to get up or go on”
- Suicidal thoughts or ideations
## PATIENT HEALTH QUESTIONNAIRE-9 (PHQ-9)

Over the last 2 weeks, how often have you been bothered by any of the following problems? (Use "0" to indicate your answer)

<table>
<thead>
<tr>
<th>Problem</th>
<th>Not at all</th>
<th>Several days</th>
<th>More than half the days</th>
<th>Nearly every day</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. Little interest or pleasure in doing things</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
</tr>
<tr>
<td>2. Feeling down, depressed, or hopeless</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
</tr>
<tr>
<td>3. Trouble falling or staying asleep, or sleeping too much</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
</tr>
<tr>
<td>4. Feeling tired or having little energy</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
</tr>
<tr>
<td>5. Poor appetite or overeating</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
</tr>
<tr>
<td>6. Feeling bad about yourself — or that you are a failure or have let yourself or your family down</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
</tr>
<tr>
<td>7. Trouble concentrating on things, such as reading the newspaper or watching television</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
</tr>
<tr>
<td>8. Moving or speaking too slowly that other people could have noticed? Or the opposite — being too fidgety or restless that you have been moving around a lot more than usual</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
</tr>
<tr>
<td>9. Thoughts that you would be better off dead or of hurting yourself in some way</td>
<td>0</td>
<td>1</td>
<td>2</td>
<td>3</td>
</tr>
</tbody>
</table>

**Total Score:**

---

If you checked off any problems, how difficult have these problems made it for you to do your work, take care of things at home, or get along with other people?

- Not difficult at all
- Somewhat difficult
- Very difficult
- Extremely difficult

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Developed by Drs. Robert L. Spitzer, Janet B.W. Williams, Kurt Kroenke and colleagues, with an educational grant from Pfizer Inc. No permission required to reproduce, tabulate, display or distribute.
ARS: What symptoms do patients with NTM lung disease report as experiencing most often and as most bothersome?

A. Coughing without sputum, fatigue, shortness of breath
B. Fatigue, coughing with sputum, weight loss
C. Fatigue, coughing with sputum, shortness of breath
D. Coughing with sputum, fatigue, shortness of breath
Patients reported these three symptoms as having the most significant clinical impact on their daily lives:

- Fatigue
- Cough
- Shortness of breath
NTM Patient Experiences

- Long delays to proper diagnosis
- Long, burdensome treatment regimens
- Side effects, some permanent
- Symptoms including:
  - Hemoptysis
  - 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, NTM patients
• Circulated through NTMinfo.org, social media channels, blast emails, Social360 forum
• 465 respondents
• Results presented at April 8th FDA workshop on Development of Antibacterial Drugs for the Treatment of Nontuberculous Mycobacterial Disease
Top Symptoms Patients Experienced

- Fatigue
- Coughing with sputum
- Shortness of breath
- Coughing without sputum
- Night sweats
- Weight loss
- Coughing up blood
- Lack of appetite
- Chest pain
- Anxiety
Most Bothersome Symptoms

- Fatigue
- Coughing up sputum
- Shortness of breath
- Coughing without sputum
- Night sweats
- Weight loss
- Lack of/ decreased appetite
- Coughing up blood
- Chest Pain
- Anxiety
Side Effects of Antibiotic Treatment

- Diarrhea
- Weight Loss
- Cognitive Dysfunction
- Dysphonia
- Nausea
- Dyspnea
- Decreased Appetite
- Tinnitus
- Cough
- Dry Mouth
- Fatigue

Ranked #1
Selected
FDA Workshop Nov. 18-19, 2019: Exploring the Clinical Trial Enterprise for Antibacterial Drug Development in the United States

• Co-sponsored by FDA, IDSA, and Pew Charitable Trusts
• Strategies to better support clinical trials for antibiotic development
THANK YOU