3rd INTERNATIONAL WORKSHOP ON LUNG HEALTH

Asthma & COPD: converging or diverging chronicity?

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Alpha-1 antitrypsin deficiency (AATD) and Non-cystic fibrosis bronchiectasis (NCFBE) survey

Monaco, 16th January 2016
DETAILS OF THE STUDY (1)

AIM OF THE STUDY:
The aim of the survey is to learn the physicians practices and experiences related to Alpha-1 antitrypsin deficiency (AATD) and Non-Cystic fibrosis bronchiectasis (NCFBE).

CONTENTS OF THE STUDY:

- Alpha-1 antitrypsin deficiency (AATD):
  - AATD testing
  - Augmentation therapy treatment
  - Interest in learning more about AATD

- Non-Cystic Fibrosis Bronchiectasis (NCFBE):
  - NCFBE patients
  - Pseudomonas aeruginosa colonization in NCFBE patients
  - NCFBE and antibiotic perception
  - Interest in learning more about NCFBE disease and patient management
DETAILS OF THE STUDY (2)

TARGET:
Doctors interested in attending the 3rd International Lung Congress 2016

FIELDWORK DATES:
From 2nd to 18th December 2015

METHODOLOGY:
Online self-completion questionnaire

QUESTIONNAIRE LENGTH:
8 minutes

UNIVERSE
352
individuals opened the newsletter sent by the organization

SAMPLE
134
opened the survey webpage

63
respondents participated in the survey (14 partial answers)

49
full completion questionnaires

RESPONSE RATE
13.9%
The countries with more individuals participating in the survey are Germany and Romania.

Q1. In what country do you mainly practice?
The vast majority of the respondents have more than 10 years of clinical experience and are mainly located at office or hospital.

**Sample profile**

<table>
<thead>
<tr>
<th>Physician location</th>
<th>Years of clinical experience</th>
<th>Number of COPD patients treated per month</th>
</tr>
</thead>
<tbody>
<tr>
<td>Hospital</td>
<td>&lt; 2: 6%</td>
<td>&lt;5: 14%</td>
</tr>
<tr>
<td>Office</td>
<td>2-10: 25%</td>
<td>6-20: 32%</td>
</tr>
<tr>
<td>Laboratory</td>
<td>11-20: 40%</td>
<td>21-50: 32%</td>
</tr>
<tr>
<td>Other</td>
<td>21-35: 25%</td>
<td>&gt;50: 22%</td>
</tr>
<tr>
<td></td>
<td>&gt; 35: 3%</td>
<td></td>
</tr>
</tbody>
</table>

Base: (63)

Q.2. Where are you primarily located?
Q.3. How many years of clinical experience do you have?
Q.4. How many COPD patients do you treat per month?
Along with Cystic Fibrosis, AATD is the disease with the least current level of experience while respondents have the highest expertise with COPD.

### Level of experience in diagnosing and treating these diseases

<table>
<thead>
<tr>
<th>Disease</th>
<th>1 (none)</th>
<th>2</th>
<th>3</th>
<th>4</th>
<th>5</th>
<th>6</th>
<th>7 (significant)</th>
<th>Mean</th>
</tr>
</thead>
<tbody>
<tr>
<td>Non-Cystic Fibrosis Bronchiectasis (NCFBE)</td>
<td>11%</td>
<td>2%</td>
<td>8%</td>
<td>5%</td>
<td>19%</td>
<td>22%</td>
<td>38%</td>
<td>5.38</td>
</tr>
<tr>
<td>Idiopathic Pulmonary Fibrosis (IPF)</td>
<td>11%</td>
<td>8%</td>
<td>14%</td>
<td>8%</td>
<td>16%</td>
<td>17%</td>
<td>25%</td>
<td>4.63</td>
</tr>
<tr>
<td>Alpha-1 Antitrypsin Deficiency (AATD)</td>
<td>16%</td>
<td>14%</td>
<td>16%</td>
<td>13%</td>
<td>16%</td>
<td>8%</td>
<td>17%</td>
<td>3.92</td>
</tr>
<tr>
<td>Cystic Fibrosis (CF)</td>
<td>22%</td>
<td>16%</td>
<td>11%</td>
<td>21%</td>
<td>11%</td>
<td>6%</td>
<td>13%</td>
<td>3.52</td>
</tr>
<tr>
<td>Emphysema</td>
<td>5%</td>
<td>3%</td>
<td>10%</td>
<td>13%</td>
<td>19%</td>
<td>51%</td>
<td></td>
<td>5.73</td>
</tr>
<tr>
<td>COPD</td>
<td>5%</td>
<td>2%</td>
<td>5%</td>
<td>5%</td>
<td>6%</td>
<td>19%</td>
<td>59%</td>
<td>5.98</td>
</tr>
</tbody>
</table>

**Q.5** Please rate your current level of experience in diagnosing and treating the following diseases? (Where 1 is none and 7 is significant: please select one option for each disease.)
Alpha-1 antitrypsin deficiency (AATD)
Epidemiology of AATD

• AATD may be among the most common hereditary disorders in the world

Prevalence of alpha-1 in the United States compared to other rare diseases

- ~ 1 in 9000 in the USA
- ~ 1 in 5000 in Europe

AAT is an “anti-enzyme” for Neutrophil Elastase

Functional AAT

Deficient or non-functional

Neutrophil elastase burden

Anti-neutrophil protection

AAT

Neutrophil elastase burden

Anti-neutrophil protection

AAT

Tissue damage from excess NE can result from inadequate AAT function

Adapted from Köhnlein T, Welte T. *Alpha-1 Antitrypsin Deficiency: Clinical Aspects and Management.* Bremen, Germany: UNI-MED Verlag AG; 2007.
Deficient or Abnormal AAT Cannot Protect the Lung

**Deficient**

Abnormal AAT does not effectively enter circulation, is not produced or is otherwise non-functional

1. Neutrophil Elastase released into lung as part of pro-inflammatory immune response

2. Insufficient A1AT enters lung

3. Available A1AT binds and destroys some Elastase

4. Undestroyed excess Elastase remains in the lung

5. Elastase irreparably damages lung tissue, possibly resulting in eventual emphysema

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Lungs and liver diagram simple.svg licensed under creative commons attribution 2.5 generic license (creative credits Patrick J. Lynch, medical illustrator; C. Carl Jaffe, MD, cardiologist). Heart Diagram, A1AT and NE schematics licensed under Creative Commons Attribution-Share Alike 3.0 Unported (no creative credits available)
Review of Alpha\textsubscript{1}-Antitrypsin Variants

- **M variant**
  - Most common variant
  - AAT function and serum concentrations are normal

- **S variant**
  - Plasma levels slightly reduced
  - Minimal clinical relevance

- **Z variant**
  - Protein misfolding leads to AAT polymerization
  - Plasma levels greatly reduced
  - One of the most common deficiency variants

- **Null variant**
  - There is no measurable AAT in the serum (lack of synthesis)

A level of less than 11 $\mu$M (80 mg/dL if measured by radial immunodiffusion; 50 mg/dL if measured by nephelometry) is associated with an increased risk for emphysema.
Age, Smoking History, or Severity of $\text{FEV}_1$ Decline Should **NOT** Define Which COPD Patients to Test

Remember that only a laboratory test can confirm the presence of alpha-1 antitrypsin deficiency

ATS/ERS guidelines recommend testing all COPD patients

<table>
<thead>
<tr>
<th>№</th>
<th>Recommendation</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>Confirmation of absent alpha-1 antitrypsin peak on serum protein electrophoresis</td>
</tr>
<tr>
<td>2</td>
<td>Early-onset pulmonary emphysema (regardless of smoking history)</td>
</tr>
<tr>
<td>3</td>
<td>Family members of known alpha-1 antitrypsin–deficient patients</td>
</tr>
<tr>
<td>4</td>
<td>Dyspnea and cough occurring in multiple family members in same or different generations</td>
</tr>
<tr>
<td>5</td>
<td>Liver disease of unknown cause</td>
</tr>
<tr>
<td>6</td>
<td>All subjects with chronic obstructive pulmonary disease</td>
</tr>
<tr>
<td>7</td>
<td>Adults with bronchiectasis without evident etiology should be considered for testing</td>
</tr>
<tr>
<td>8</td>
<td>Patients with asthma whose spirometry fails to return to normal with therapy</td>
</tr>
<tr>
<td>9</td>
<td>Unexplained panniculitis and anti–proteinase-3 vasculitis</td>
</tr>
</tbody>
</table>

Adapted from: ATS/ERS Guidelines

For analysis purposes, countries have been separated related to Augmentation therapy reimbursement

<table>
<thead>
<tr>
<th>Augmentation therapy is reimbursed</th>
<th>Augmentation therapy is NOT reimbursed</th>
</tr>
</thead>
<tbody>
<tr>
<td>Austria</td>
<td>Albania</td>
</tr>
<tr>
<td>Brazil</td>
<td>Malta</td>
</tr>
<tr>
<td>Canada</td>
<td>Algeria</td>
</tr>
<tr>
<td>Colombia</td>
<td>Moldova</td>
</tr>
<tr>
<td>Germany</td>
<td>Belgium</td>
</tr>
<tr>
<td>Greece</td>
<td>Poland</td>
</tr>
<tr>
<td>Portugal</td>
<td>Bosnia-Herzegovina</td>
</tr>
<tr>
<td>Spain</td>
<td>Romania</td>
</tr>
<tr>
<td>Switzerland</td>
<td>Bulgaria</td>
</tr>
<tr>
<td>USA</td>
<td>Russian Federation</td>
</tr>
<tr>
<td></td>
<td>Denmark</td>
</tr>
<tr>
<td></td>
<td>Serbia</td>
</tr>
<tr>
<td></td>
<td>India</td>
</tr>
<tr>
<td></td>
<td>Tunisia</td>
</tr>
<tr>
<td></td>
<td>Ireland</td>
</tr>
<tr>
<td></td>
<td>Turkey</td>
</tr>
<tr>
<td></td>
<td>Latvia</td>
</tr>
<tr>
<td></td>
<td>Ukraine</td>
</tr>
<tr>
<td></td>
<td>Lithuania</td>
</tr>
<tr>
<td></td>
<td>United Kingdom</td>
</tr>
<tr>
<td></td>
<td>Macedonia</td>
</tr>
</tbody>
</table>
The vast majority (71%) test for AATD currently, although the percentage is higher (82%) in those countries where the augmentation therapy is reimbursed.

**Current testing for AATD (% of respondents)**

- Augmentation therapy is reimbursed:
  - Yes: 82%
  - No: 9%
  - Not Sure: 9%

- Augmentation therapy is NOT reimbursed:
  - Yes: 65%
  - No: 33%
  - Not Sure: 3%

**Base:** Sample (62)
- Augmentation therapy is reimbursed (22)
- Augmentation therapy is not reimbursed (40)

**Q.6.** Do you currently test for Alpha-1 Antitrypsin Deficiency (AATD)?
The main reason for not testing for AATD is that physicians think that serum level test is too expensive. There is also opportunity to improve awareness of AATD testing methods.

### Reasons for not testing for AATD

<table>
<thead>
<tr>
<th>1st top reasons</th>
<th>2nd top reasons</th>
<th>3rd top reasons</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>AUGMENTATION THERAPY IS REIMBURSED</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Testing Alpha-1 Protein serum level is too expensive (25%)</td>
<td>Testing Alpha-1 Protein serum level is too expensive (50%)</td>
<td>Testing Alpha-1 Protein serum level is too expensive (25%)</td>
</tr>
<tr>
<td>I am not aware of the testing methods (25%)</td>
<td>Patients refuse testing (25%)</td>
<td></td>
</tr>
<tr>
<td><strong>AUGMENTATION THERAPY IS NOT REIMBURSED</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Augmentation therapy (Alpha-1 Proteinase Inhibitor replacement via infusion) for AATD doesn't exist in my country, so there is no reason to test (36%)</td>
<td>Testing Alpha-1 Protein serum level is too expensive (36%)</td>
<td>It takes too long to get test results and to follow up with patients (29%)</td>
</tr>
<tr>
<td>Testing Alpha-1 Protein serum level is too expensive (36%)</td>
<td>Patients refuse testing (21%)</td>
<td>My peers do not recommend testing (21%)</td>
</tr>
</tbody>
</table>

*Base: Augmentation therapy is reimbursed (4)*

*Caution low base*

*Base: Augmentation therapy is not reimbursed sample (14)*

Q.7. If you do not currently test for AATD (answered "no" in Question 6), what are the top 3 reasons why you do not test? (Please Pick 3)
It seems that do not believe in augmentation therapy is not a reason for not testing at all

Reasons for not testing for AATD (2)

Nobody mentioned the following reason for not testing in any country:

- I do not believe augmentation therapy is beneficial, so there is no reason to diagnose AATD
In general, respondents perform between none and 5 tests per month. Most physicians have tested less than 10% of their COPD patients in all countries.

### AATD tests performed per month

- **None**: 18% (Augmentation therapy is reimbursed: 9%; Augmentation therapy is not reimbursed: 37%)
- **1-5**: 64% (Augmentation therapy is reimbursed: 8%; Augmentation therapy is not reimbursed: 55%)
- **6-10**: 14% (Augmentation therapy is reimbursed: 14%; Augmentation therapy is not reimbursed: 8%)
- **>10**: 5% (Augmentation therapy is reimbursed: 5%; Augmentation therapy is not reimbursed: 5%)

### Percentage of COPD patients ever tested for AATD

- **None**: 9% (Augmentation therapy is reimbursed: 9%; Augmentation therapy is not reimbursed: 24%)
- **<10%**: 41% (Augmentation therapy is reimbursed: 3%; Augmentation therapy is not reimbursed: 50%)
- **11-20%**: 18% (Augmentation therapy is reimbursed: 5%; Augmentation therapy is not reimbursed: 18%)
- **21-30%**: 3% (Augmentation therapy is reimbursed: 3%; Augmentation therapy is not reimbursed: 3%)
- **31-40%**: 5% (Augmentation therapy is reimbursed: 5%; Augmentation therapy is not reimbursed: 3%)
- **41-50%**: 5% (Augmentation therapy is reimbursed: 5%; Augmentation therapy is not reimbursed: 5%)
- **61-70%**: 3% (Augmentation therapy is reimbursed: 3%; Augmentation therapy is not reimbursed: 3%)
- **71-80%**: 5% (Augmentation therapy is reimbursed: 5%; Augmentation therapy is not reimbursed: 5%)
- **>80%**: 9% (Augmentation therapy is reimbursed: 9%; Augmentation therapy is not reimbursed: 9%)
Serum AAT level test in a hospital lab is most often the first step in testing. Genotyping/phenotyping is rarely done as a first step.

**Initial Test for AATD**

<table>
<thead>
<tr>
<th>Option</th>
<th>Augmentation therapy is reimbursed</th>
<th>Augmentation therapy is not reimbursed</th>
</tr>
</thead>
<tbody>
<tr>
<td>Order only a serum AAT level from a Hospital Lab</td>
<td>29%</td>
<td>39%</td>
</tr>
<tr>
<td>Order only a serum AAT level from somewhere other than a Hospital Lab</td>
<td>18%</td>
<td>29%</td>
</tr>
<tr>
<td>Use a Dry Blood Spot only for genotyping</td>
<td>9%</td>
<td>3%</td>
</tr>
<tr>
<td>Use a Dry Blood Spot sample for both the serum AAT level and genotyping/phenotyping</td>
<td>5%</td>
<td>5%</td>
</tr>
<tr>
<td>Order a serum AAT level (whole blood) and use a Dry Blood Spot for genotyping, at the same visit</td>
<td>5%</td>
<td>5%</td>
</tr>
<tr>
<td>Other (Please specify)</td>
<td>5%</td>
<td>3%</td>
</tr>
<tr>
<td>I do not test for AATD</td>
<td>14%</td>
<td>26%</td>
</tr>
</tbody>
</table>

Q.10. How do you most often do the initial test for AAT Deficiency?

Base: Sample (60)
- Augmentation therapy is reimbursed (22)
- Augmentation therapy is not reimbursed (38)
Young COPD patients (<45 y.o.) and those with early onset Emphysema are most often tested for AATD. Patients with adult-onset asthma, COPD patients over 60 y.o., and COPD patients who are not of European descent or not 100% Caucasian are less likely to get tested.

Q. 11. Using a scale of 1 to 7, where 1=never and 7=always, please indicate how often you test each of the following patient types for AATD?

- All COPD
- Young COPD (<45 yrs)
- Early onset Emphysema
- COPD patients over the age of 60
- Family members of diagnosed AATD patients
- COPD patients under age 50 who currently smoke
- Adult-onset Asthma
- Bronchiectasis
- COPD patients who have never smoked
- COPD patients who are not of Western or Eastern European descent
- COPD patients who are not 100% Caucasian

Augmentation therapy is reimbursed
Augmentation therapy is not reimbursed
About half of the respondents from countries with AAT reimbursement have never prescribed augmentation therapy while the percentage increases dramatically where it is not reimbursed.

Q.12. Have you ever prescribed augmentation (Alpha-1 Proteinase Inhibitor replacement) therapy for patients with AATD?
In the countries with AAT reimbursement, roughly 75% of physicians have less than 5 severe AATD patients while where there is not reimbursement the percentage is about 85%.

Severe AATD patients care

- Augmentation therapy is reimbursed (Mean: 5.49)
- Augmentation therapy is not reimbursed (Mean: 2.22)

Q.13. How many patients with severe AAT deficiency do you currently have under your care?

Base: Sample (56)
- Augmentation therapy is reimbursed (20)
- Augmentation therapy is not reimbursed (36)
Some respondents believe the augmentation therapy is not available in their countries even it is (*). 30% of physicians have all diagnosed AATD patients on augmentation therapy.

**Percentage of severe AATD patients are NOT on augmentation therapy**

- **0% (they are all on augmentation therapy)**: 30%
- **<25%**: 20%
- **26-50%**: 15%
- **51-75%**: 10%
- **76-99%**: 10%

100% not on therapy because it is not available in my country

100% not on therapy for other reasons: 22%

(**) In some countries without AAT reimbursement, respondents say that there are some severe AATD patients on augmentation therapy: Bosnia-Herzegovina, Romania, Turkey, Macedonia, Russian Federation, UK, India, Poland and Tunisia

**Base:** Sample (56)
- Augmentation therapy is reimbursed (20)
- Augmentation therapy is not reimbursed (36)

Q.14. What percent of your patients with severe AAT deficiency are NOT on augmentation therapy?
The main reasons for not treating with augmentation therapy where it is reimbursed are that the patient doesn’t meet requirement for reimbursement (FEV1 too high) or is still smoking.

### Reasons for not treating for AATD

<table>
<thead>
<tr>
<th>1st top reasons</th>
<th>2nd top reasons</th>
<th>3rd top reasons</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>AUGMENTATION THERAPY IS REIMBURSED</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Patient doesn’t meet requirement for reimbursement - FEV1 too high (35%)</td>
<td>Patient still smoking (30%)</td>
<td>Patient doesn't meet requirement for reimbursement - FEV1 too low (25%)</td>
</tr>
<tr>
<td>Patient still smoking (20%)</td>
<td>Patient doesn't meet requirement for reimbursement - FEV1 too high (25%)</td>
<td>Patient doesn't meet requirement for reimbursement - FEV1 too high (20%)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Patients don't want infusion treatment (20%)</td>
</tr>
<tr>
<td><strong>AUGMENTATION THERAPY IS NOT REIMBURSED</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>My hospital administration severely restricts the number of patients I can put on therapy (31%)</td>
<td>Local experts do not support treatment (30%)</td>
<td>Local experts do not support treatment (31%)</td>
</tr>
<tr>
<td>Local experts do not support treatment (23%)</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

**Base:** Augmentation therapy is reimbursed sample (16)  
Augmentation therapy is not reimbursed sample (17)  

Q.15. If <100% of severe AATD patients are on augmentation therapy, the main reasons for not treating are that the patient doesn’t meet requirement for reimbursement (FEV1 too high) or is still smoking.
Where augmentation therapy is reimbursed, respondents have most interest in counseling and long-term management of AATD patients.

**AATD interest (Mean)**

- **How to get access to alpha-1 replacement therapy (augmentation therapy)**
  - Augmentation therapy is reimbursed (Mean: 4.5, Standard Deviation: 6.5)
  - Augmentation therapy is not reimbursed (Mean: 6.5, Standard Deviation: 4.0)

- **Counseling and long-term management of AATD patients**
  - Augmentation therapy is reimbursed (Mean: 5.5, Standard Deviation: 6.5)
  - Augmentation therapy is not reimbursed (Mean: 6.5, Standard Deviation: 4.0)

- **Safety of alpha-1 replacement therapy (augmentation therapy)**
  - Augmentation therapy is reimbursed (Mean: 4.7, Standard Deviation: 6.6)
  - Augmentation therapy is not reimbursed (Mean: 6.6, Standard Deviation: 4.0)

- **Efficacy of alpha-1 replacement therapy (augmentation therapy)**
  - Augmentation therapy is reimbursed (Mean: 5.3, Standard Deviation: 6.6)
  - Augmentation therapy is not reimbursed (Mean: 6.6, Standard Deviation: 4.0)

- **How to treat AATD**
  - Augmentation therapy is reimbursed (Mean: 5.1, Standard Deviation: 6.7)
  - Augmentation therapy is not reimbursed (Mean: 6.7, Standard Deviation: 4.0)

- **Who should be treated for AATD**
  - Augmentation therapy is reimbursed (Mean: 4.9, Standard Deviation: 6.7)
  - Augmentation therapy is not reimbursed (Mean: 6.7, Standard Deviation: 4.0)

- **GOLD Guidelines recommendation for testing AATD in COPD...**
  - Augmentation therapy is reimbursed (Mean: 4.2, Standard Deviation: 6.6)
  - Augmentation therapy is not reimbursed (Mean: 6.6, Standard Deviation: 4.0)

- **Who should be tested for AATD**
  - Augmentation therapy is reimbursed (Mean: 4.7, Standard Deviation: 6.4)
  - Augmentation therapy is not reimbursed (Mean: 6.4, Standard Deviation: 4.0)

- **How to diagnose AATD**
  - Augmentation therapy is reimbursed (Mean: 4.4, Standard Deviation: 6.2)
  - Augmentation therapy is not reimbursed (Mean: 6.2, Standard Deviation: 4.0)

- **Prevalence of AATD**
  - Augmentation therapy is reimbursed (Mean: 4.3, Standard Deviation: 5.9)
  - Augmentation therapy is not reimbursed (Mean: 5.9, Standard Deviation: 4.0)

- **Pathophysiology of AATD**
  - Augmentation therapy is reimbursed (Mean: 5.9, Standard Deviation: 5.9)
  - Augmentation therapy is not reimbursed (Mean: 5.9, Standard Deviation: 4.0)

**Base:** Sample (53)
- Augmentation therapy is reimbursed (19)
- Augmentation therapy is not reimbursed (34)

**Q.16.** On a scale of 1 to 7 (with 1=no interest at all and 7=extremely high interest), how interested are you in learning more about AATD disease, testing/diagnosis and patient treatment/management?
Bronchiectasis

Recurrent cough, sputum and respiratory infections

Common-reported prevalence of 52/100,000

Failed bacterial clearance with chronic bacterial colonisation and neutrophilic airway inflammation

The cause is unknown in >60% of cases

No licensed therapies- Historically neglected
Aetiology
Why should COPD experts and researchers care about bronchiectasis?

N=3636
Bronchiectasis
20.8% - associated with more exacerbations, worse FEV$_1$

N=2164
Bronchiectasis
5% GOLD III, 7% GOLD IV

Single centre studies
• 50-60% of patients with moderate to severe COPD
• More bacterial colonisation
• More *P. aeruginosa*
• Independent predictor of death

Stewart et al, AJRCCM 2012; Agusti et al, Respir Res 2012; Martinez et al AJRCCM 2013; Getheral et al COPD 2014
Why should COPD experts and researchers care about bronchiectasis?

- 750 million people in Europe
- 5-10% have COPD
- 5-50% of these have bronchiectasis
- A conservative estimate suggests at least 1m people in Europe have COPD associated bronchiectasis

Figure 2  Kaplan–Meier log-rank test survival curve per NCFB etiology over the study period: There was a median follow-up time of 5.18 years and the study period started in June 2006 and ended in November 2013. COPD = Chronic Obstructive Pulmonary Disease; NCFB = Non-cystic fibrosis bronchiectasis.
The vast majority have treated ≤10 NCFBE patients in the last 6 months. Almost half of the respondents report that the percentage of NCFBE patients colonized with *Pseudomonas aeruginosa* is ≤20%.

**Number of NCFBE patients treated in the last 6 months**

<table>
<thead>
<tr>
<th>Number of Patients</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>0-10 patients</td>
<td>71%</td>
</tr>
<tr>
<td>11-20 patients</td>
<td>8%</td>
</tr>
<tr>
<td>21-30 patients</td>
<td>6%</td>
</tr>
<tr>
<td>31-40 patients</td>
<td>2%</td>
</tr>
<tr>
<td>41-50 patients</td>
<td>4%</td>
</tr>
<tr>
<td>51-200 patients</td>
<td>8%</td>
</tr>
</tbody>
</table>

**% of NCFBE patients colonized with *Pseudomonas aeruginosa***

<table>
<thead>
<tr>
<th>Percentage of Colonization</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>0-10%</td>
<td>18%</td>
</tr>
<tr>
<td>11-20%</td>
<td>27%</td>
</tr>
<tr>
<td>21-30%</td>
<td>16%</td>
</tr>
<tr>
<td>31-40%</td>
<td>6%</td>
</tr>
<tr>
<td>41-50%</td>
<td>4%</td>
</tr>
<tr>
<td>51-60%</td>
<td>8%</td>
</tr>
<tr>
<td>61-70%</td>
<td>4%</td>
</tr>
<tr>
<td>71-80%</td>
<td>2%</td>
</tr>
<tr>
<td>81-90%</td>
<td>6%</td>
</tr>
<tr>
<td>91-100%</td>
<td>2%</td>
</tr>
</tbody>
</table>

I don’t treat any patients with NCFBE: 45%

Q.17. How many NCFBE patients have you treated in the last 6 months? (Please consider each individual as “1 patient” even if seen multiple times)

Q.18. What percent of your NCFBE patients are colonized with *Pseudomonas aeruginosa*?
Almost half of the respondents report that the percentage of their NCFBE patients colonized with *Pseudomonas aeruginosa* treated with chronic antibiotic is 20% or below.

**Q.19.** Of your patients colonized with *Pseudomonas aeruginosa*, what percent are on chronic antibiotic treatment (any type of antibiotic)?

- **0-20%**: 49%
- **21-40%**: 4%
- **41-60%**: 16%
- **61-80%**: 10%
- **81-100%**: 14%
- **I don’t treat any patients with NCFBE**: 6%

*Base: (49)*
The perceived NCFBE prevalence seems to be staying about the same or increasing somewhat.

**Perception of the NCFBE prevalence change**

- Decreasing somewhat: 8%
- Staying about the same: 43%
- Increasing somewhat: 47%
- Increasing dramatically: 2%

Q.20. How do you believe the prevalence of NCFBE is changing in your country?
Physicians believe that every severe exacerbation in NCFBE causes a permanent decline in the patient’s overall well-being/quality of life and lung function. There is some lack of comfort with chronic use of antibiotics and apparent concern about development of resistance with inhaled antibiotics.

**Perceptions of NCFBE and antibiotics**

(1=Completely disagree, 7=Completely agree)

- The risk of antibiotic resistance developing with long-term use of inhaled antibiotics is very low: **3.63**
- I am very comfortable prescribing antibiotics for chronic use for my NCFBE patients: **4.04**
- I am very comfortable prescribing antibiotics for acute use for my NCFBE patients: **4.51**
- Every severe exacerbation in NCFBE causes a permanent decline in the patient's overall well-being/quality of life: **5.83**
- Every severe exacerbation in NCFBE causes a permanent decline in the patient's lung function: **5.59**
- A significant percentage of NCFBE cases are idiopathic: **4.57**

Base: (49)

Q.21. On a scale of 1 to 7 (where 1=completely disagree and 7=completely agree), please rate your level of agreement with the following statements:
Interest in knowing more about…
Respondents are highly interested in learning more about AATD and NCFBE diseases, patient treatment and management.

### Interest in learning more about...

<table>
<thead>
<tr>
<th>AATD</th>
<th>NCFBE disease and patient treatment/management</th>
</tr>
</thead>
<tbody>
<tr>
<td>Pathophysiology of AATD</td>
<td>7 - extremely high interest</td>
</tr>
<tr>
<td>Prevalence of AATD</td>
<td>67%</td>
</tr>
<tr>
<td>How to diagnose AATD</td>
<td>7 - extremely high interest</td>
</tr>
<tr>
<td>Who should be tested for AATD</td>
<td>6 - high interest</td>
</tr>
<tr>
<td>GOLD Guidelines recommendation for...</td>
<td>5 - moderate interest</td>
</tr>
<tr>
<td>Who should be treated for AATD</td>
<td>4 - low interest</td>
</tr>
<tr>
<td>How to treat AATD</td>
<td>3 - no interest at all</td>
</tr>
<tr>
<td>Efficacy of alpha-1 replacement therapy...</td>
<td>2 - no interest at all</td>
</tr>
<tr>
<td>Safety of alpha-1 replacement therapy...</td>
<td>1 - no interest at all</td>
</tr>
<tr>
<td>Counseling and long-term management...</td>
<td></td>
</tr>
<tr>
<td>How to get access to alpha-1...</td>
<td></td>
</tr>
</tbody>
</table>

**Base:**
- AATD (63)
- NCFBE disease and patient treatment/management (49)

**Q.22.** On a scale of 1 to 7 (with 1 = no interest at all and 7 = extremely high interest), how interested are you in learning more about NCFBE disease and patient treatment/management?
What is EMBARC?

• A pan-European collaborative network to promote research in bronchiectasis
• Funded and supported by the European Respiratory Society as a clinical research collaboration
• An alliance between national networks, expert centres and investigator
• **Open to everyone**
EMBARC promotes awareness and clinical excellence in bronchiectasis care through educational events, courses and online resources.

EMBARC is a pan-European network committed to promoting clinical research and education in bronchiectasis, through sharing of protocols, research idea and expertise. Central to this project is the creation of the European Bronchiectasis Registry, a collaboration open to all investigators around Europe caring for patients with bronchiectasis.

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Sign up at the registration page

Follow EMBARC on Facebook!
What can we achieve with a European Bronchiectasis Registry?
Data from 1310 patients in 4 countries
The first validated prediction rule for bronchiectasis
What EMBARC needs to achieve

• Better understanding of the natural history of bronchiectasis
• Understanding the impact of disease phenotypes
• Promote a higher profile for bronchiectasis research
• Facilitate Clinical Trials
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