

Alpha-1 antitrypsin deficiency (AATD) Patient survey

EARCO CRC

Data comparison:

Responses from those living in countries with AATD therapy reimbursement and those living in countries where AATD therapy is not reimbursed

May 2020

RESPONSES FROM THOSE LIVING IN COUNTRIES WITH AATD THERAPY REIMBURSEMENT AND THOSE LIVING IN COUNTRIES WHERE THERAPY IS NOT REIMBURSED

About the respondents

124 survey responses from people living in the following non-reimbursed countries: Australia, Denmark, Finland, Ireland, Norway, Sweden and United Kingdom were compared with 239 survey responses from people living in the following reimbursed countries: Argentina, Austria, France, Germany, Italy, Portugal, Spain, Switzerland and United States of America.

Who were the respondents?

Non-reimbursed: 86% (n=107) identified as a person diagnosed with AATD and 14% (n=17) identified as a parent, relative or caregiver of someone with AATD.

Reimbursed: 81% (n=194) identified as a person diagnosed with AATD and 19% (n=45) identified as a parent, relative or caregiver of someone with AATD.

Gender

Non-reimbursed: 63% female (n=78), 35% male (n=44) and 2% prefer not to say (n=2)

Reimbursed: 55% female (n=132), 45% male (n=107).

Age

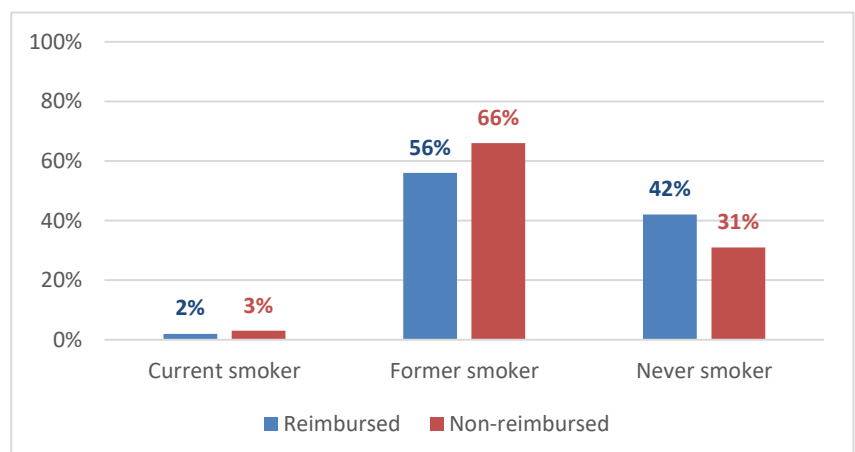
Non-reimbursed: respondents were aged between 1 and 82 years with the mean age of respondents 54 years and the median age 52 years.

Reimbursed: respondents were aged between 1 and 82 years with the mean age 52 years and the median age 48 years.

Smoking

Non-reimbursed (n=119): 66% (n=78) were a former smoker, 31% (n=27) never smoker, 3% (n=4) current smoker.

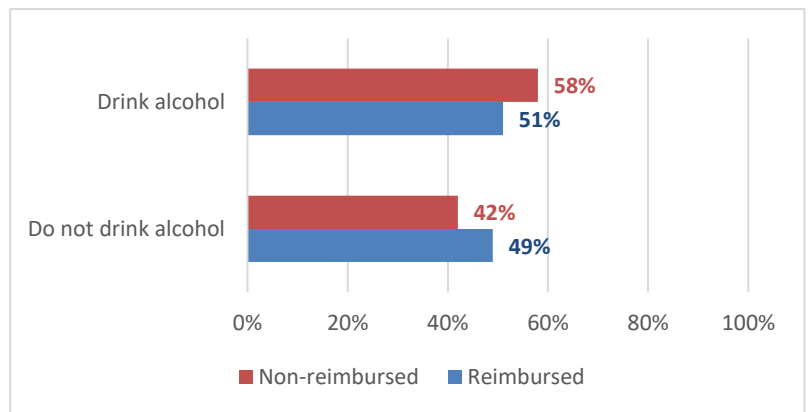
Reimbursed (n=225): 56% (n=126) were a former smoker, 42% (n=94) never smoker, 2% (n=5) current smoker.



Alcohol

Non-reimbursed (n=119): 58% (n=69) drink alcohol and 42% (n=50) do not drink.

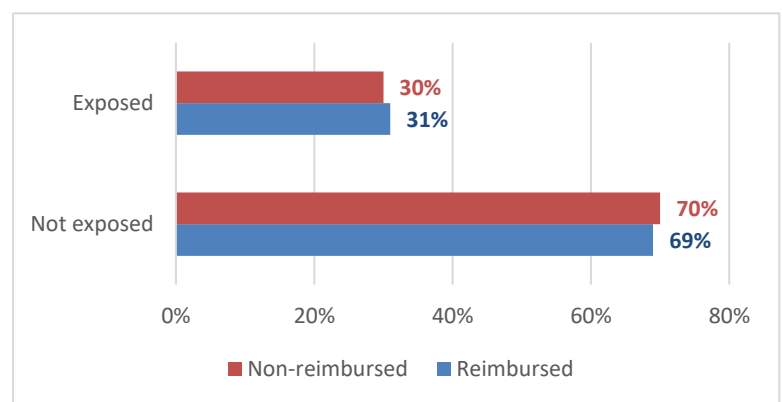
Reimbursed (n=222): 51% (n=113) drink alcohol and 49% (n=109) do not drink.



Environmental exposure

Non-reimbursed (n=119): 70% (n=83) said No they were not exposed to gases, fumes or dust in their professional activities and 30% (n=36) said Yes they were.

Reimbursed (n=225): 69% (n=156) said No they were not exposed to gases, fumes or dust in their professional activities and 31% (n=69) said Yes they were.



Transplants

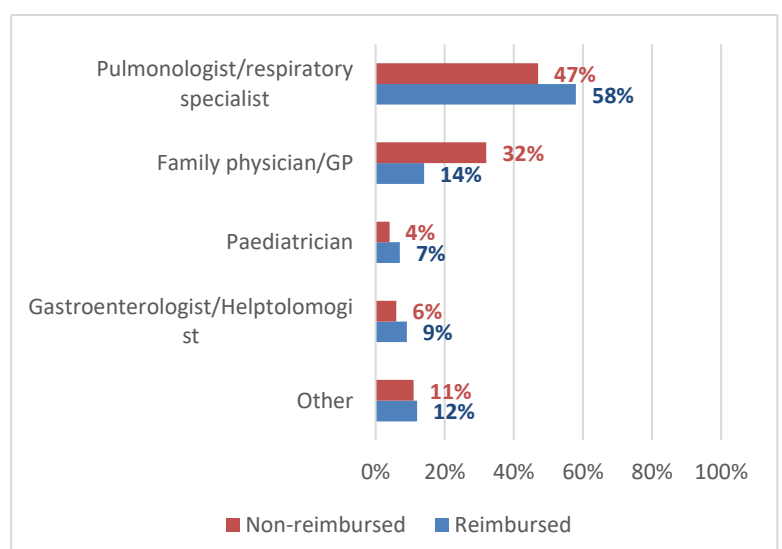
Non-reimbursed (n=119): 1% (n=1) of respondents had a liver transplant and 2% (n=2) respondents had a lung transplant.

Reimbursed (n=225): 1% (n=3) of respondents had a liver transplant and 1% (n=3) had a lung transplant.

Who diagnosed you?

Non-reimbursed (n=119): 47% (n=56) Pulmonologist/respiratory specialist; 32% (n=38) Family physician/GP; 6% (n=7) Gastroenterologist; 4% (n=5) Paediatrician; 11% (n=13) Other.

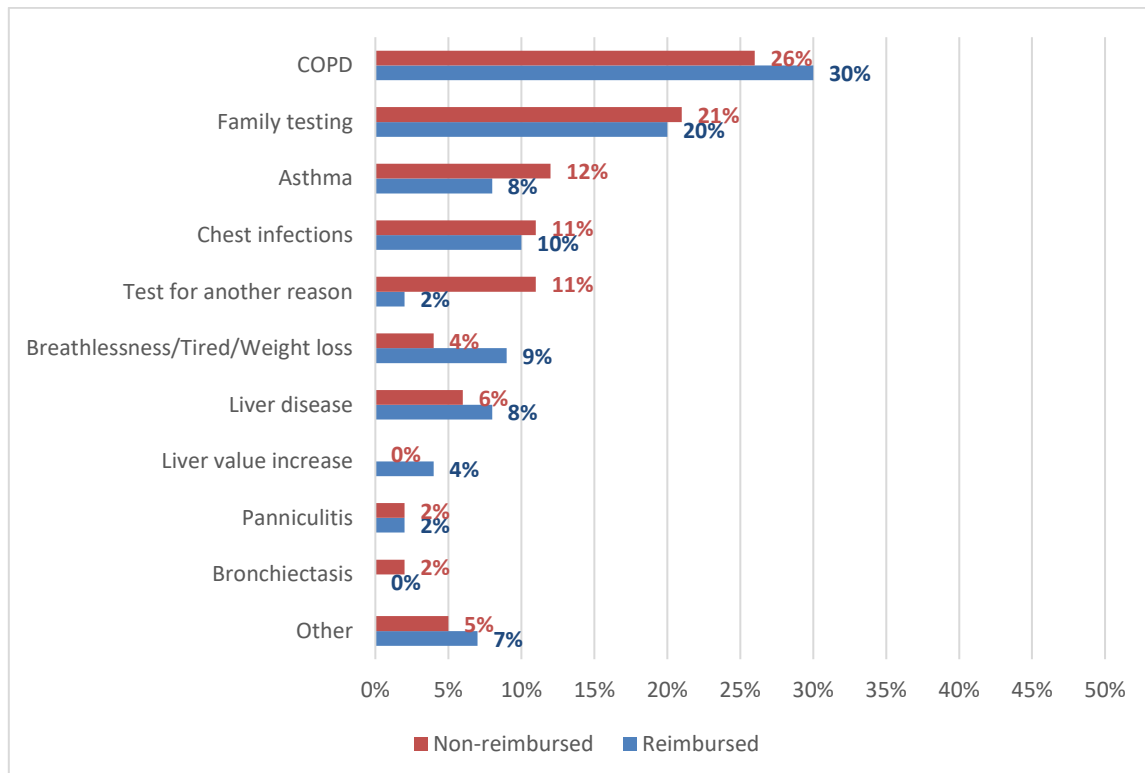
Reimbursed (n=214): 58% (n=125) Pulmonologist/respiratory specialist; 14% (n=29) Family physician/GP; 9% (n=19) Gastroenterologist; 7% (n=14) Paediatrician; 12% (n=27) stated Other.



Reason for diagnosis

Non-reimbursed (n=119): The three most common reasons for diagnosis were: 26% (n=31) COPD; 21% (n=25) Family testing; 12% (n=14) Asthma.

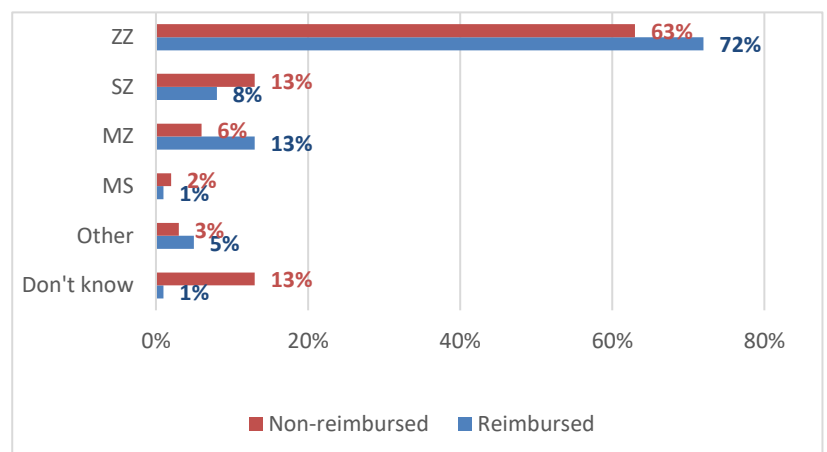
Reimbursed (n=225): The three most common reasons for diagnosis were: 30% (n=68) COPD; 20% (n=45) Family testing; 10% (n=23) Chest infections.



Phenotype

Non-reimbursed (n=119): Most respondents 63% are ZZ (n=75)

Reimbursed (n=224): Most respondents 72% are ZZ (n=161)



How long since diagnosis?

Non-reimbursed (n=119): The length of time since diagnosis ranged from 'less than 1 year' to 45 years with the mean length being 10 years and the median length being 6 years.

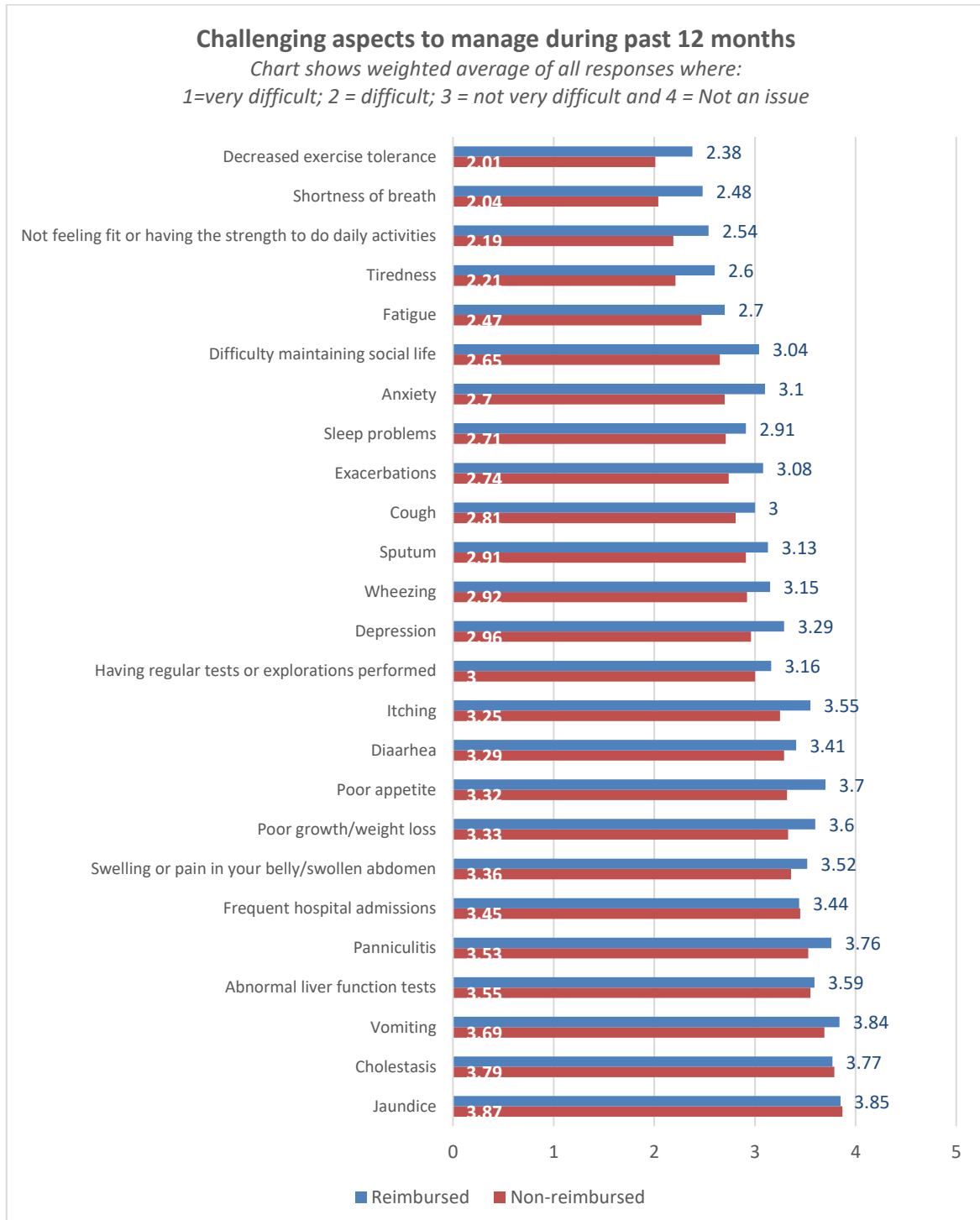
Reimbursed (n=222): The length ranged from 'less than 1 year' to 59 years with the mean length being 11 years and the median length 8 years.

Your experience of AATD

Most challenging aspects to manage during the past 12 months.

Non-reimbursed: 1. Decreased exercise tolerance 2. Shortness of breath 3. Not feeling fit or have strength for daily activities

Reimbursed: 1. Decreased exercise tolerance 2. Shortness of breath 3. Not feeling fit for activities



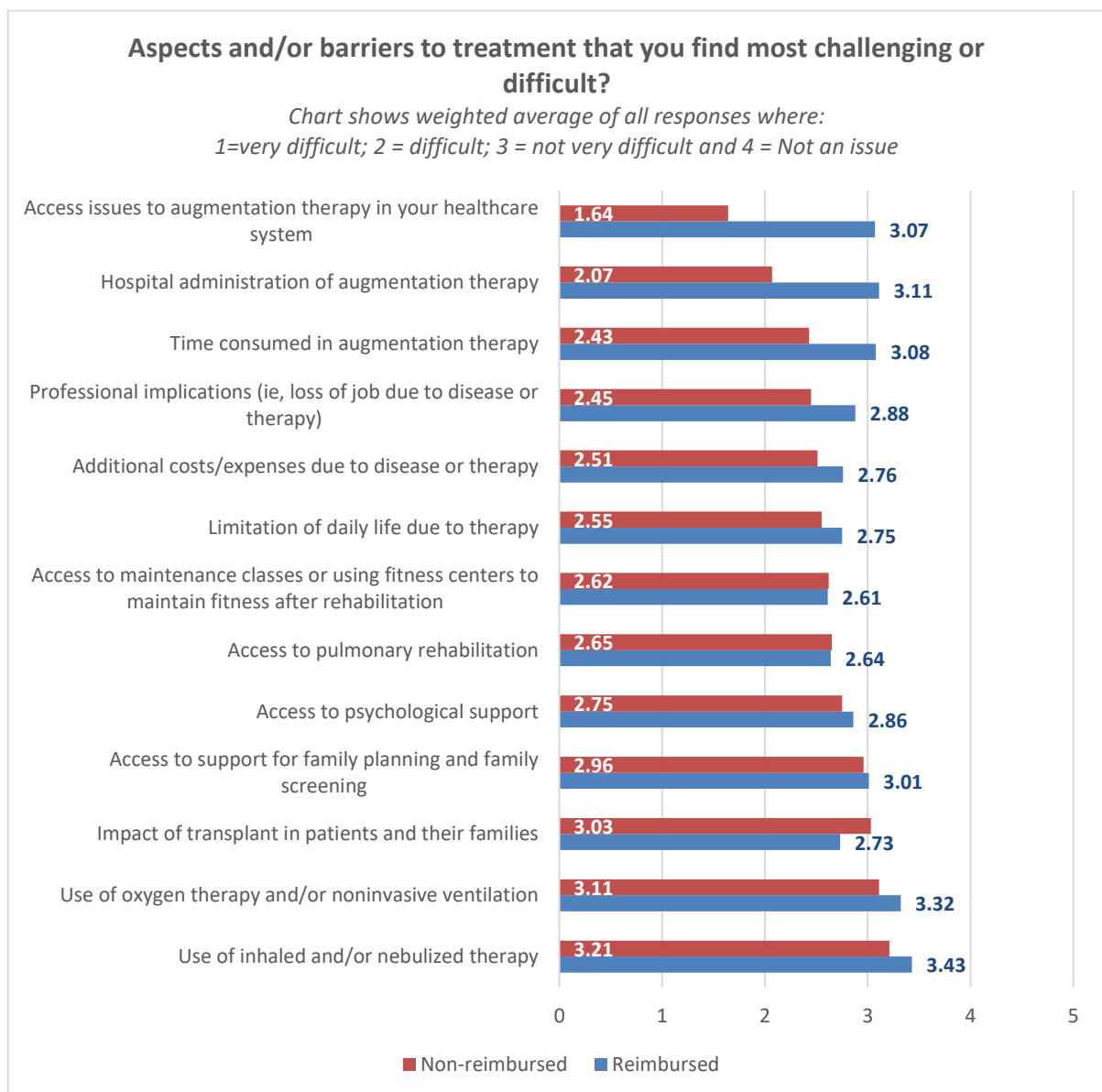
Most challenging aspects/barriers for treatment

Non-reimbursed:

1. Access issues to augmentation therapy in your healthcare system (1.64)
2. Hospital administration of augmentation therapy (2.07)
3. Time consumed in augmentation therapy (2.43)

Reimbursed:

1. Access to maintenance classes / fitness centers to maintain fitness after rehabilitation (2.61)
2. Access to pulmonary rehabilitation (2.64)
3. Impact of transplant in patients and their families (2.73)



Research prioritisation

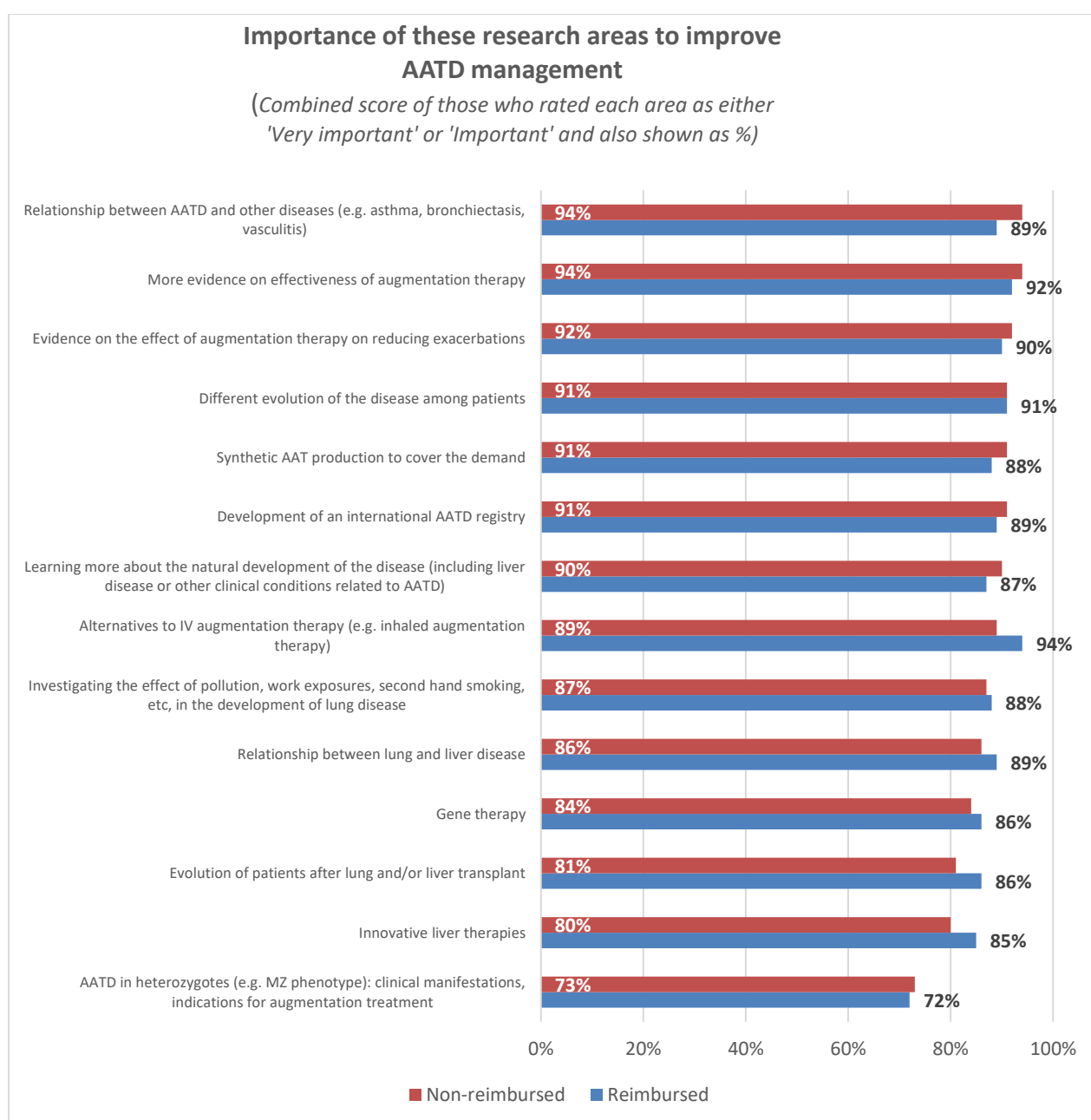
Improving AATD management - The most important research areas:

Non-reimbursed:

1. Relationship between AATD and other diseases (94%)
2. More evidence on effectiveness of Aug Therapy (94%)
3. Evidence on the effect of augmentation therapy on reducing exacerbations (92%)

Reimbursed:

1. Alternatives to IV augmentation therapy (94%)
2. More evidence on effectiveness of augmentation therapy (92%)
3. Different evolution of the disease among patients (91%)



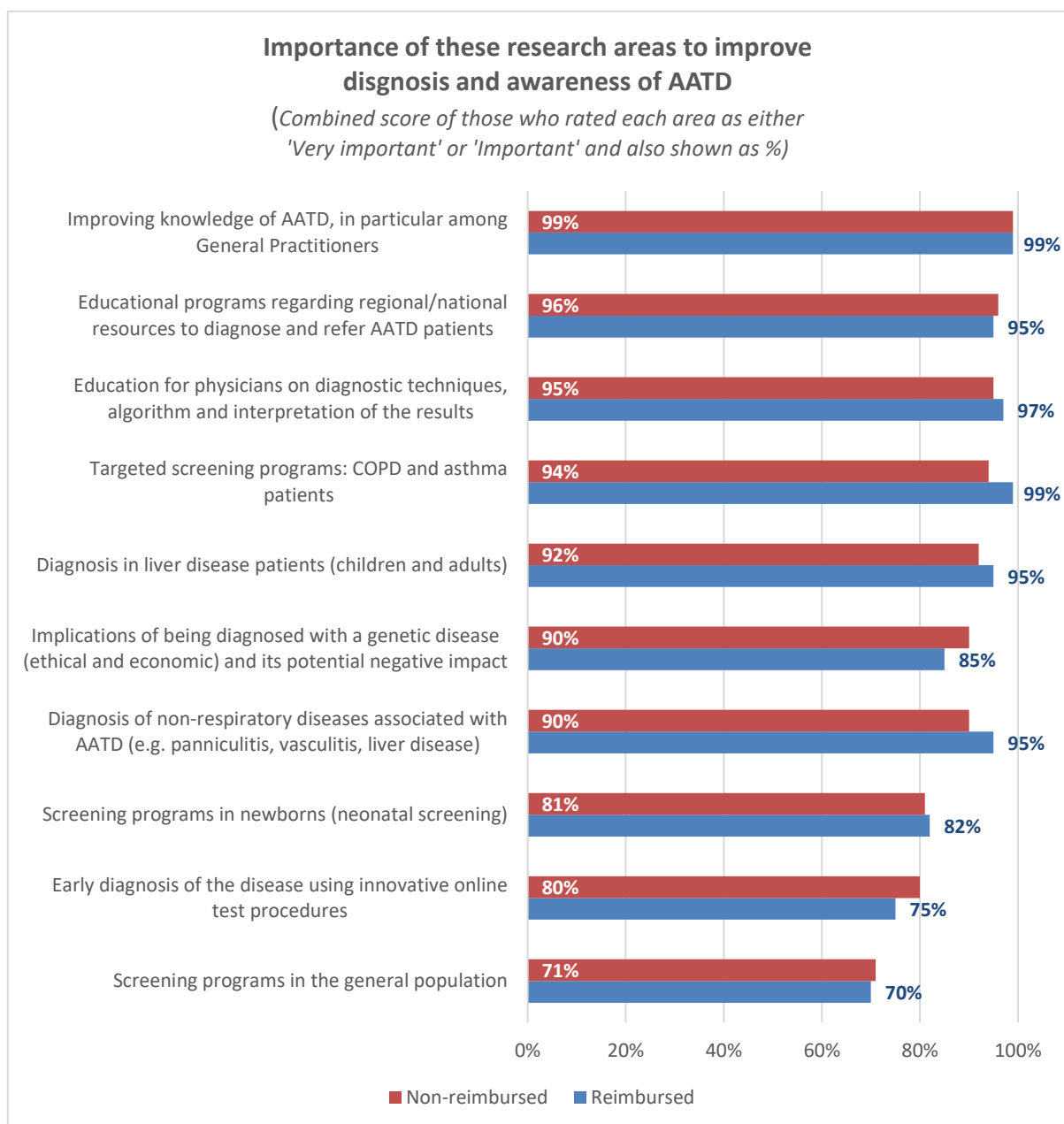
Improving diagnosis and awareness of AATD

Non-reimbursed:

1. Improving knowledge of AATD in particular among GPs (99%)
2. Educational programs for regional/national resources to diagnose/refer patients (96%)
3. Education for physicians on diagnostic techniques, algorithm & results (95%)

Reimbursed:

1. Improving knowledge of AATD in particular among GPs (99%)
2. Targeted screening programs: COPD and Asthma patients (99%)
3. Education for physicians on diagnostic techniques, algorithm & results (97%)



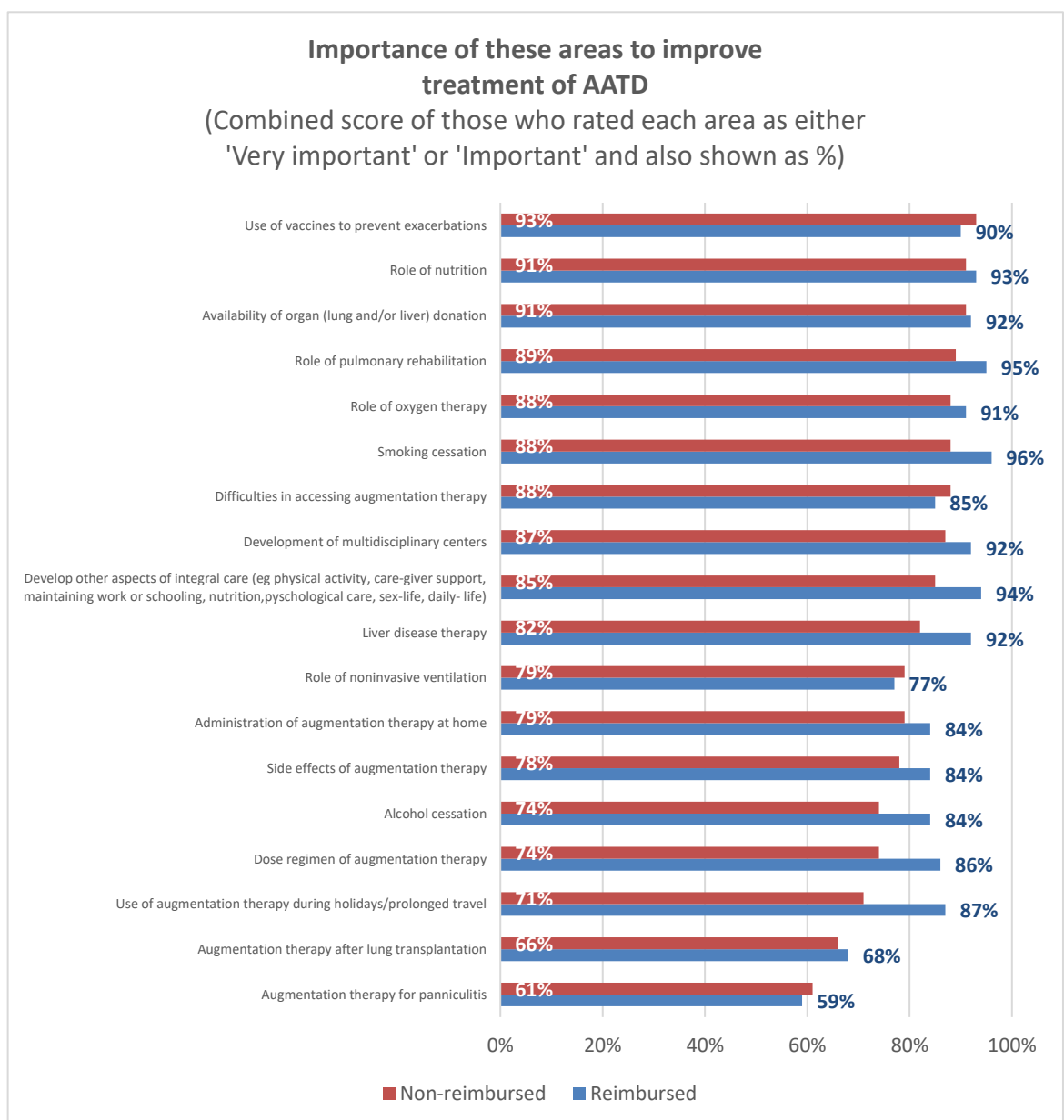
How important do you think it is to improve the following areas for AATD treatment?

Non-reimbursed:

1. Use of vaccines to prevent exacerbations (93%)
2. Role of nutrition / Availability of organ donation (both 91%)
3. Role of pulmonary rehabilitation (89%)

Reimbursed:

1. Smoking cessation (96%)
2. Role of pulmonary rehabilitation (95%)
3. Develop other aspects of integral care (94%)



Self-management and education

Non-reimbursed:

1. Having access to AATD specialized centres (99%)
2. Having access to reliable, easy to understand information about AATD (99%)
3. Having an action plan for exacerbations and easy access to healthcare (99%)

Reimbursed:

1. A personalised integrated care plan including therapeutic physical activity (98%)
2. Being able to recognise an exacerbation (97%)
3. Regular communication between healthcare professional and AATD patient (97%)

