

# Research Priorities in $\alpha$ 1-Antitrypsin Deficiency: Results of a Patients' and Healthcare Providers' International Survey from the EARCO Clinical Research Collaboration

Barrecheguren et al. (2020)  
<https://doi.org/10.1183/23120541.00523-2020>

## For which topic were research priorities identified?

Alpha1-antitrypsin deficiency (AATD)

## In which location was the research priority setting conducted?

international

## Why was it conducted at all?

$\alpha$ 1-antitrypsin deficiency (AATD) is a rare and under-recognised genetic condition. Owing to its low prevalence, international initiatives are key for conducting high-quality research in the field.

## What was the objective?

to identify research priorities and barriers in access to treatment for  $\alpha$ 1-antitrypsin deficiency (AATD)

## What was the outcome?

a ranking list of 20 research questions

## How long did the research prioritization take?

June 2018 - December 2019

## Which methods were used to identify research priorities?

survey

## How were the priorities for research identified exactly?

Step 1: survey: one survey among healthcare providers and one survey among patients and their caregivers, list of 164 research questions, participants asked to rate each question

## Which stakeholders took part?

Patients, parents, relatives and caregivers and AATD experts. 533 participants: 368 patients: 70 parents/relatives/caregivers, 94 AATD experts (healthcare providers: 92.6% pulmonologists)

## How were stakeholders recruited?

Participants were amongst others AATD experts.

## Were stakeholders actively involved or did they just participate?

Stakeholders not only participated but were also actively involved in the research prioritization process: They were part of an advisory board. The advisory board consisted of 2 advisory groups: one formed by five EARCO members, one formed by four expert patients with AATD.