

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

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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?

α 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 α 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.