

Research Award:

Patient-centred composite indices for assessment of response to biological therapy for paediatric and adult severe asthma

Awarded to: Dr Ekaterina Khaleva **Amount:** £7,664 (May 2022 – May 2023)

Lay summary

Patients with severe asthma have ongoing symptoms, impaired well-being, and risk of severe asthma attacks despite modern medicines. Biologics are injection medicines that are effective for these patients. However, there is no agreement about what counts as 'responding' to biologics— for example, how much better a patient should feel.

This study aimed to (1) find out what aspects of asthma (eg asthma symptoms, quality of life) patients and clinicians consider to be most important and (2) develop a single patient-centred measure of response to biologics that takes into account all the aspects of asthma severity. The developed tool is called CompOsite iNdex For Response in asthMa (CONFIRM).

The project involved 146 participants from across the world including patients with severe asthma, clinicians, health regulators and pharmaceutical representatives. They rated the extent to which example patient descriptions have improved with biologic treatment. Subsequently, they decided which of two different example patients has improved most. Highest relative importance in CONFIRMs for children and adults with severe asthma in the assessment of response to biologics were assigned to severe asthma attacks and regular oral corticosteroids use. There was strong agreement between clinicians and patients, but patients assigned greater importance to quality of life. Both CONFIRMs have showed good initial validity meaning the tool measures what it supposed to measure. Further, both CONFIRMs had excellent discriminative ability for substantial and sufficient response to biologics. Substantial was defined as an improvement in asthma that a patient would consider as being 'big enough' to justify the continued use of the biologic therapy; a sufficient response was smaller, defined as the smallest improvement in asthma that a patient would consider as important. CONFIRM will allow clinicians to make patient-centred decisions about which biologics should be licensed and which should be used in individual patients. This is especially important due to rapidly increasing number of biologics for severe asthma. Lastly, using CONFIRM will help to ensure that the measures are used to make decisions in clinical trials of biologics are patient-centred and improve patient's outcomes.

I have submitted the results of this project to the journal and aim to present this work at several european and international conferences. This manuscript will form part of my PhD thesis at the University of Southampton. In future work, I am planning to validate the CONFIRMs using ongoing prospectively collected data in several clinical studies.

Khaleva E, Brightling C, Eiwegger T, et al. Patient-centred composite indices for assessment of response to biological therapy for paediatric and adult severe asthma. Under review.
