Title: DO PATIENTS WITH CYSTIC FIBROSIS PARTICIPATING IN CLINICAL TRIALS DEMONSTRATE PLACEBO RESPONSE? A META-ANALYSIS

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What was your research question?
Can people with cystic fibrosis (CF) improve while being included in the placebo arms of clinical trials (ie experiencing a placebo response)? Evaluating the placebo response in clinical trials may be of importance to accurately assess the therapeutic effect of a given drug.

Why is this important?
People with CF and their families have high expectations that a cure for CF will be found. In some diseases, a similar high expectation has been shown to induce a strong response to placebo during clinical trials.

What did you do?
We collected data from placebo-controlled CF clinical trials and studied the change of three clinically relevant outcome measures at the beginning and at the end of the trial duration. The outcomes respectively assessed lung function, nutritional status and patient’s quality of life.

What did you find?
Out of 289 clinical trials identified, 61 reported one of the three outcomes at the beginning and the end of the trial. We did not find any evidence of a placebo response when looking at
the lung function or quality of life outcomes. They both tended to deteriorate along the trial period. However, a small improvement of the nutritional outcome was observed which could potentially be related to a placebo response.

What does this mean and reasons for caution?
The deterioration of both lung function and quality of life outcome measures can be explained by the progressive nature of the CF disease. Nutritional status improvement could be explained by a placebo response, but it may also be related with the normal increase of BMI with age of patients with CF, particularly in children.

What’s next?
Studying individual data from patients included in placebo arms would help us understand the reasons for the small placebo response observed on the nutritional status outcome.

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