Abstract

Cystic fibrosis is an inherited disease that primarily affects the respiratory system but also the gastrointestinal tract. Nutritional status is a significant factor in overall health and prevention of malnutrition is a key component of therapy. New modulator therapy contributes to major improvement in health status as well as quality and length of life.

The aim of the bachelor thesis was to evaluate the development of nutritional status after initiating modulator therapy. The study assessed whether there was an increase in BMI, whether the treatment response was affected by the initial nutritional status, and whether the percentage of patients with target and better nutritional status increased.

The research sample consisted of 86 patients from the Cystic Fibrosis Centre at the Motol University Hospital. The development of BMI was evaluated based on retrospective collection of anthropometric data. Data from 4 measurements were used: before treatment and then at 1, 3 and 6 months after starting treatment. Friedman test and comparison of median values were conducted to evaluate BMI development. Patients were divided into 4 groups to verify whether the initial nutritional status affects the treatment response. One-way ANOVA or the Kruskal-Wallis test were used to assess statistically significant difference in the average improvement in BMI between the groups. The last part examined the percentage of each group of patients divided by nutritional status in each period.

The results confirmed that the modulator therapy leads to an increase in BMI. The greatest changes in the evaluated sample were observed in patients with malnutrition. However, the results did not demonstrate statistically significant difference in the average improvement in BMI values between the groups. The percentage of patients with target and better nutritional status increased from 45 % to 62 %.

The study confirmed that with the new modulator therapy, the number of patients with malnutrition decreases, but there is also an increase in the number of patients with overweight and obesity. The results indicate the necessity to update nutritional recommendations for patients with cystic fibrosis which have been primarily focused on preventing and addressing malnutrition. The study also highlights potential cardiometabolic risk factors associated with extending the life expectancy and increasing the BMI of patients.

Keywords: cystic fibrosis, nutritional status, CFTR modulators