Other interventions

Growth hormone therapy

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Background

Normal growth is essential for good clinical conditions of CF patients. Unfortunately, despite careful nutrition protocols, growth often remains below normal values and often there is a delay both in puberty onset and in attaining adult height according to the family target (Lucidi V, 2009). This condition is mainly due to respiratory impairment and malabsorption, however it has been hypothesized (D'Orazio C, 2012) that Growth Hormone (GH) release impairment may be an independent cause, because the pituitary gland itself might be damaged by CF disease and/or there is an intrinsic defect in the growth that may be ameliorated by CFTR modulation (Stalvey MS, 2017), (Le Tn, 2019)

GH is the most active human effector for growth, stimulating the production of the Insulin-like growth factor-1 (IGF-1) in the liver, which is the most important substance for protein synthesis. IGF-1 serum levels, that are low in CF patients also because of the associated inflammatory cytokine over-production, result in loss of lean body mass and respiratory muscle wasting (Wong SC, 2016).

Recombinant human GH (rh-GH) has been available since 1985 (Wilson TA, 2003) and it proved to be effective in improving growth in patients affected by diseases other than CF, as idiopathic short stature, Turner syndrome, Prader-Willi syndrome, chronic renal insufficiency, small for gestational age children.

A rhGH possible role in CF has been hypothesized (Hardin DS, 2008) (Lavi E, 2020) . However, as it is affirmed in ESPEN-ESPGHAN-ECFS guidelines on nutrition care for infants, children, and adults with cystic fibrosis (Turck D, 2016), further studies are required before growth hormone can be recommended.

Issues

rh-GH therapy efficacy on nutritional condition, lung function, bone mineralization, pubertal onset, exercise tolerance, pulmonary exacerbation frequency, quality of life, survival.

rh-GH therapy safety (among possible adverse events: glucose intolerance, benign intracranial hypertension, malignant disease).

CF candidates for rh-GH therapy.

Efficacy and safety of rh-GH therapy compared to other therapies.

What is known

One Cochrane review (Thaker V, 2018) including 5 RCTs and a more recent one (Thaker V, 2021), including 8 studies, concluded that when compared with no treatment, rhGH therapy is effective in improving the intermediate outcomes in height, weight and lean body mass. Some measures of pulmonary function showed moderate improvement, but no consistent benefit was seen across all trials. The significant change in blood glucose levels, although not causing diabetes, emphasizes the need for careful monitoring of this adverse effect in a population predisposed to CF-related diabetes.

Unresolved questions

rh-GH safety and efficacy in long-term studies, also compared to other intervention strategies.

No RCT are ongoing on this topic.

Keywords

Failure to Thrive;