JOURNAL CLUB

Thalidamide for IPF-associated cough

This small, randomised twelve-week crossover trial based at Johns Hopkins in Baltimore, USA, assessed the efficacy of thalidomide versus placebo on cough in patients with idiopathic pulmonary fibrosis (IPF). This drug when previously given as Contergan for hyperemesis gravidarum led to birth defects; however, it is now approved for the treatment of refractory multiple myeloma and other malignant diseases.

After performing a phase 2 trial (published in 2008), the authors enrolled 25 mild-moderate IPF patients (20 completed) suffering from cough adversely affecting their quality of life. The Cough Quality of Life Questionnaire (CQLQ) was the primary outcome comparing CQLQ scores in those taking placebo and those on thalidomide. The CQLQ has 28 items on a 4-point Likert scale. The mean score improved from 60.5 (12.0) to 58.7 (14.0) under placebo, and to 47.2 (13.4) after treatment with thalidomide 50–100 mg od; treatment difference –11.4 (–15.7 to –7.0). This is a large and significant effect. Secondary outcomes included significant improvements of the cough VAS score of –31.2 points and SGRQ total score –11.7 points; a value far above the minimal clinically important difference (MCID) of 4 units, seldom achieved in pharmacological trials.

The large effect size is spectacular, relieving frequently debilitating and intractable cough in IPF. The side effects of the 50–100 mg daily were mild. However, no additional objective cough recording—as suggested in cough assessment guidelines—was performed. Moreover, the Leicester cough questionnaire with established MCID could have also been a useful tool. As yet, no data on the effect of thalidomide on IPF itself are available.

► Horton MR, Santopietro V, Mathew L, et al. Thalidomide for the treatment of cough in idiopathic pulmonary fibrosis. Ann Int Med 2012:57:398—406.

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