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New treatment option may be on the horizon for polymyalgia rheumatica

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A drug approved to treat rheumatoid arthritis, tocilizumab (Actemra, Genentech), is a potential new therapy for patients with polymyalgia rheumatica, according to an open-label, phase II study presented at the annual meeting of the American College of Rheumatology/Association of Rheumatology Health Professionals on November 10. The inflammatory disorder impacts 1% of people over the age of 50 and is predominantly found in individuals over the age of 65.

Polymyalgia rheumatica causes muscle pain and stiffness, especially in the shoulders, and can also cause people to feel like they have the flu, with mild fever, fatigue, and malaise. Corticosteroids are the current treatment option, but they have downsides.

"Treatment often requires a long course of steroid therapy, often up to two years, which can have serious side effects in the older population. Steroids can cause skin fragility, diabetes, osteoporosis, cognitive disturbances, and muscle weakness, so there is a desire to identify a drug that can help patients get off steroids more quickly," said lead author Robert Spiera, MD, director of the Vasculitis and Scleroderma Program at Hospital for Special Surgery (HSS), in New York City. "This study is a proof of concept, and it strongly suggests that this drug is efficacious and confers a steroid sparing effect."

Tocilizumab is a medication designed to specifically block the cytokine interleukin-6, a protein that is involved in various inflammatory disorders. Studies have shown that patients with polymyalgia rheumatica

have elevated levels of interleukin-6, and thus researchers at HSS decided to test whether tocilizumab could help this patient population.

The HSS investigators enrolled 10 subjects with newly diagnosed polymyalgia rheumatica who had received less than one month of treatment with corticosteroids. Patients received tocilizumab once a month by intravenous infusion in addition to corticosteroids, but patients were tapered off the steroids within four months, much more quickly than is done in routine clinical practice.

One subject withdrew after two months due to an infusion reaction, leaving nine subjects in whom the primary endpoint was assessed. All of these patients achieved the primary endpoint of the trial, relapse-free remission and no longer taking corticosteroids at six months. Of the nine subjects who have been evaluated for 12 months to date, all remain in remission without relapse. All eight of the patients who have reached 15 months of followup are still in remission.

As a control group, the investigators followed10 consecutively diagnosed patients with polymyalgia rheumatica patients with similar baseline characteristics who were treated with only corticosteroids. In this group, no patients were in remission off of corticosteroids at six months, and 60% had relapsed at 12 months.

The cumulative corticosteroid dose in the control group was more than twice that in the tocilizumab group (2,562 mg vs. 1,085 mg). Eight of the nine patients treated with tocilizumab were able to discontinue corticosteroids following the third dose of tocilizumab, and the ninth patient tapered off following the fourth dose. Tocilizumab was well tolerated.

The HSS researchers are hoping that their findings will lead to a placebo-controlled trial to provide additional evidence that the drug works for

polymyalgia rheumatica. If a trial confirms the drug's efficacy, the drug's price may be a barrier to it being incorporated in clinical practice. "It is expensive, like all of our biologics," said Dr. Spiera. "If cost were not an issue though, I think it could potentially be a favored way of approaching this disease if the safety and efficacy suggested in our open label trial is confirmed in a larger controlled study."

Because cost is an issue in the United States health care system, the drug, if given a new indication, may be reserved for select patients. "Occasionally in practice, we see people who are having a very hard time getting off steroids, because they have refractory disease, or we see people in whom you know that steroids are going to be particularly problematic. Examples include patients with diabetes, osteoporosis with multiple fractures in the past, or they are very frail. This proof of concept trial suggests that this could be a strategy in those selected patients to minimize their steroid exposure."

Multiple interleukin-6 inhibitors are in the drug pipeline, and competition could bring down the price of this class of drugs. In addition, the price might not be such a roadblock if researchers could prove that the cost of the drug was less than the aggregate costs of treating patients for the side effects of corticosteroids, such as a compression fracture or a hip fracture, said Dr. Spiera.

"If our findings are confirmed in a placebo-controlled trial, and if a large study suggested that the aggregate costs were less using this new drug, then the drug could be relevant for more people," said Dr. Spiera.

Provided by Hospital for Special Surgery

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