Gene-Targeting Therapy Shows Promise Against Hepatitis C

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New clinical data from the early stage Phase II study demonstrate that four out of nine participants treated with the highest dose of miravirsen—7 milligrams per kilogram (mg/kg) of body weight—saw their HCV viral loads decrease to undetectable levels with just four weeks of treatment.

These findings, said study presenter Harry Janssen, MD, PhD, of Erasmus MC University Hospital in Rotterdam, Netherlands, suggest that miravirsen's unique mechanism-of-action offers a high barrier to viral resistance and the potential for cure, used either alone or with other medications. He also noted that miravirsen was well tolerated in people living with HCV, signaling a possible advantage over today's standard pegylated interferon–based treatment.

MicroRNAs are small strands of genetic material found in the human genome. In recent years, they've been discovered to have all sorts of functions in the body. Some are vital to life, whereas others, including miR-122, have been found to be more trouble than they're worth for some people, notably those living with HCV infection.

By targeting and sequestering miR-122, a liver-specific microRNA that is considered critical for HCV's genetic material to overwhelm liver cells, miravirsen has the indirect effect of profoundly reducing the amount of disease-causing virus in the organ.

The randomized, double-blind, placebo-controlled, multiple-dose clinical trial was primarily designed to evaluate the safety and tolerability of miravirsen in people living with genotype 1 HCV who had yet to begin treatment. Study volunteers were allotted to one of three groups exploring miravirsen at doses of 3 mg/kg, 5 mg/kg and 7 mg/kg—nine patients in each group received active drug; three received placebo. All patients were given a total of five weekly injections over 29 days.

According to Janssen's report, miravirsen's activity is long-lasting. Ten weeks after participants began weekly treatment with miravirsen—about five weeks after the last injection was given—HCV viral loads remained substantially reduced by 0.57, 2.16 and 2.73 log copies below pre-therapy levels in those who received 3, 5 7 mg/kg miravirsen, respectively. These viral load reductions were statistically significant—too great to have occurred by chance—when compared to the unchanged HCV viral loads documented in the placebo recipients.

There were no severe adverse events in the study. The most frequent side effects, which tended to be mild-to-moderate in intensity, were headache, upper respiratory tract inflammation and diarrhea. No significant changes to laboratory values were documented.

“Miravirsen, the first microRNA targeted therapy to be administered to patients, was well tolerated and showed continuous and prolonged antiviral activity well beyond the end of active therapy,” Janssen noted in his concluding remarks. “Miravirsen has the potential to be a once-weekly treatment of chronic HCV infection. Further trials in combination with direct acting antiviral agents are planned.”

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