Romiplostim more effective than standard care for immune thrombocytopenia

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A new study finds that an FDA-approved drug to treat the rare autoimmune disorder immune thromobocytopenia (ITP) is more effective than earlier medical therapies in helping patients avoid surgical treatment and significantly improving their quality of life. The paper in the Nov. 11 *New England Journal of Medicine* reports that treatment with romiplostim, which mimics the effects of a growth factor that regulates platelet production, was more than three times more successful than standard therapy with steroids or immunosuppressive drugs.

"This is the first definitive study with this type of medication showing that patients experienced significant clinical improvements, not just increased platelet levels," says David Kuter, MD, DPhil, of the Massachusetts General Hospital (MGH) Cancer Center, lead and corresponding author of the study. "It confirms that treatment really makes a difference for patients beyond improving platelet counts."

In ITP, which affects about one in 10,000 individuals, the immune system produces antibodies against platelets, blood cells that are essential to clot formation. Platelets are targeted for destruction in the spleen, putting patients at risk for uncontrolled bleeding. While the condition in children usually resolves within 6 months, in three-quarters of affected adults it becomes chronic. To prevent serious bleeding episodes, patients may seriously curtail their lifestyle, avoiding physical activity and medical procedures like colonoscopy and dental extractions.

Medical treatment for ITP has long relied on steroids or

immunosuppressive drugs, which have significant side effects that many patients find intolerable. Most patients eventually have their spleens removed, which can raise platelet levels for up to five years; but for older patients or those with some medical conditions, splenectomy may not be safe or medically appropriate. Clinical trials of romiplostim, one of a new group of drugs that mimic the effects of the growth factor thrombopoietin, showed that it increased the platelet counts of ITP patients for up to five years, but those studies neither compared its effects to those of standard treatments nor examined the clinical relevance of the higher platelet levels.

The current study – sponsored by Amgen, which markets romiplostim under the brand name Nplate – enrolled 234 adult ITP patients at 85 sites in North America, Europe and Australia. Participants, none of whom had their spleens removed, were randomly assigned either the standard care medications prescribed by their treating physicians or weekly romiplostim injections. Participants in both groups were able to receive other therapies that became necessary. Treatment was considered to be ineffective if platelet levels remained low for four consecutive weeks or if participants experienced major bleeding events or required changes in therapy, including splenectomy.

At the end of the 52-week study period, participants receiving romiplostim achieved and maintained desirable platelet levels at more than twice the rate of those on standard care. Overall, romiplostim treatment was at least three times as effective as other treatments, and participants receiving the drug had a 90 percent reduction in the need for splenectomy. Responses to several quality-of-life survey instruments were significantly higher for participants receiving romiplostim.

"We expected that this medication would be more effective than the standard of care but were surprised at how much more effective it was," says Kuter. Several questions still need to be resolved before the place of

romiplostim in long-term treatment of ITP can be determined, he notes. One is the treatment's cost effectiveness, since it is expensive; another is whether patients receiving romiplostim can continue to avoid splenectomy for longer than a year. Additional research also needs to determine whether romiplostim treatment may be continued indefinitely.

"Our study enrolled patients at various stages of their disease – some who had recently been diagnosed, some who'd been treated for many years. Future studies need to look at whether romiplostim may be an appropriate first-line treatment for recently diagnosed patients, allowing them to completely avoid taking steroids," says Kuter, who is director of the Center for Hematology at the MGH Cancer Center and a professor of Medicine at Harvard Medical School.

Provided by Massachusetts General Hospital