Hemostatic drug less effective than originally predicted

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The use of recombinant activated factor 7 (rFVIIa) – a drug used to treat bleeding in hemophiliacs – in patients without hemophilia is not recommended because of the potential for adverse events, found a study published in CMAJ (Canadian Medical Association Journal).

Recombinant activated factor 7 is a hemostatic agent licensed for the treatment of bleeding in hemophilia patients. It is also used "off-label" for the prevention and treatment of bleeding in patients without hemophilia including patients undergoing surgery, liver transplants and other procedures.

The use of rFVIIa in people without hemophilia appears to be common. For example, out of more than 2700 cases in the Australian and New Zealand Hemostasis Registry that use rFVIIa, only 1% had a diagnosis of hemophilia.

This systematic review was conducted to analyze the effectiveness and risks of rFVIIa in patients without hemophilia and to assess the implications of these results for future research.

"Physicians must believe that 'off-label' use is effective and outweighs risks," write Dr. Yulia Lin, Sunnybrook Health Sciences Centre and coauthors. "However, randomized controlled trials (RCTs) evaluating rFVIIa have raised concerns about adverse effects, particularly thromboembolic events (blood clots)."

The review, which looked at 14 prophylactic use RCTs including 1137 patients and 12 therapeutic use RCTs with 2538 patients, found uncertainty about the benefits and harms of this therapy.

"Clinically significant benefits of rFVIIa as a more general hemostatic drug (outside of hemophilia) remain unproven," conclude the authors. "This systematic review has not shown a consistent benefit of off-label rFVIIa use in the therapeutic setting and (at best) only modest benefits in the prophylactic setting. Given the potential risks, it cannot be recommended and in most cases, its use should be restricted to a clinical trial."

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