

Monoclonal antibody BTT1023 targeting vascular adhesion protein 1 for treating primary sclerosing cholangitis: BUTEO single-arm Phase II trial

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Plain English summary

BUTEO single-arm phase II trial

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Plain English summary

Primary sclerosing cholangitis is an inflammatory and progressive liver disease. At present, there are no approved treatments for patients. The molecule vascular adhesion protein 1 has previously been shown in laboratory studies to be potentially involved in disease, including, in particular, in the development of liver scarring.

We wanted to test the idea that blocking this molecule with a drug given as an infusion would change markers of scarring in patient blood samples. To do so, we gave the drug BTT1023 (timolumab) by infusion to patients with primary sclerosing cholangitis who had elevated activity of the blood marker alkaline phosphatase. We gave the drug to 22 patients and carried out a variety of blood tests before the drug was given and after seven infusions. We followed patients for any side effects of treatment. After 18 patients had been treated, we performed an interim analysis, which showed that, although the drug appeared safe, it was not possible to show any effect of infusions on liver inflammation. As a result, the study was subsequently ended.

Final analysis of all available tests has not shown an effect of this drug on relevant blood markers of inflammation or scarring in patients with primary sclerosing cholangitis.

In conclusion, although vascular adhesion protein 1 may play a role in the development of primary sclerosing cholangitis and how the disease progresses, blocking the activity of vascular adhesion protein 1 by treating patients with the drug BTT1023 had no effect on inflammation.

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