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NCI's phase II trial of PF-03084014 in adults with desmoid tumors/ aggressive fibromatosis

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This study evaluated the efficacy of the novel the γ -secretase inhibitor PF-03084014 in patients with recurrent, refractory, progressive desmoid tumors (NCT01981551). PF-030844014 (PF) is an oral, reversible γ -secretase inhibitor that was well tolerated in a prior phase I trial. PF was administered orally at 150mg BID; 21 day cycles. Primary objective was response rate (RR). This is a single stage study; 90% power to rule out unacceptably low RR of 10% in favor of 35% RR. Response defined by RECIST 1.1 using CT. Optional dynamic contrast-enhanced MRI (DCE-MRI) was obtained at baseline and with every restaging. 17 patients entered between November 2014 and May 2015. Currently, 10/17 patients (59%) remain on study. 7 patients discontinued treatment by choice or due to co-morbid conditions, including skin allergy, desire to conceive and travel issues. Updated results will be presented, showing PF-03084014 is well tolerated and demonstrates promising clinical benefit in patients with refractory, progressive desmoid tumors treated long-term with this agent.