Summary paragraph - Is ruxolitinib the new standard for steroid refractory acute and chronic GVHD? Yes

Allogeneic hematopoietic cell transplantation (allo-HCT) is a potentially curative treatment for different hematological malignancies. A major life-threatening complication is acute graft-versus-host disease (GVHD) in particular when the disease becomes steroid refractory (SR). Based on the broad array of cytokines that contribute to GVHD pathogenesis it is conceivable that depletion of one cytokine will not prevent disease. In recent years specific inhibitors that target signaling molecules have become available. In preclinical GVHD models inhibition of Janus activates kinases (JAK) 1/2 improved outcome. Early clinical data including retrospective analyses and a prospective phase I trial using ruxolitinib for SR GVHD suggested clinical activity. If the currently active phase III trials on ruxolitinib for SR-aGVHD and SR-cGVHD will show an advantage over BAT then ruxolitinib may become a new standard for this hard to treat disease.