

CTGTAC Discussion Topic #1

Discuss whether the results of Study HOPE-3 on the pre-specified secondary endpoint of change from baseline to month 12 in left ventricular ejection fraction (LVEF) support a conclusion that deramioceol is effective in slowing the rate of decline in cardiac function in patients with DMD.

CTGTAC Discussion Topic #2

Discuss whether the results of Study HOPE-3 on the pre-specified primary and secondary endpoints assessing upper limb function and cardiac imaging parameters support a conclusion that deramiocele is effective in the treatment of patients with DMD.

CTGTAC Voting Question

The logo of the U.S. Food and Drug Administration (FDA), consisting of the letters "FDA" in white on a blue square background.

Does the available evidence provide substantial evidence of effectiveness of deramiocecl for the treatment of cardiomyopathy in patients with DMD?

Please vote “Yes” or “No” or “Abstain”