Human microRNA inhibits expression of pathogenic gene underlying facioscapulohumeral muscular dystrophy

December 14 2021

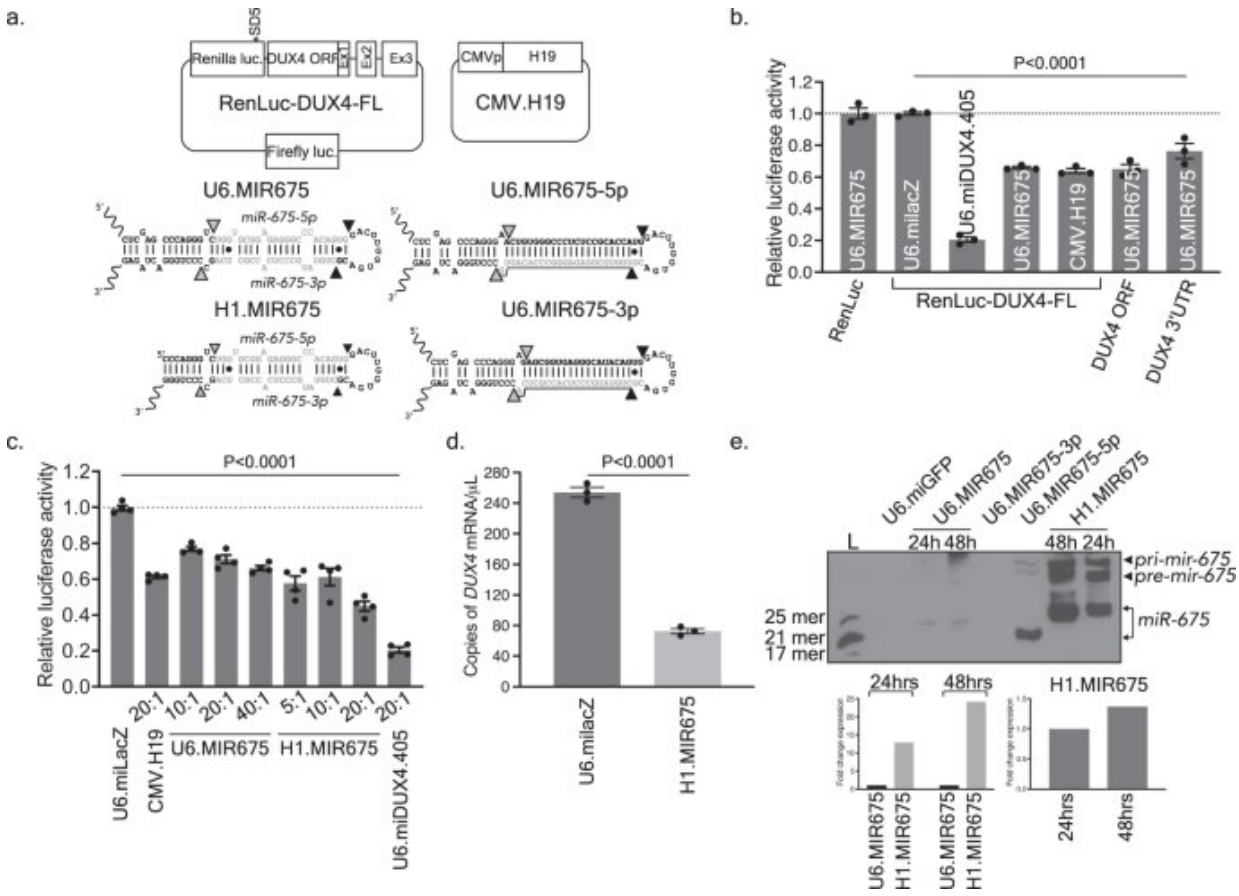

Fig. 1: miR-675 targets the DUX4 ORF and 3′UTR. a RenLuc-DUX4-FL dual-luciferase reporter and CMV.H19 constructs, and U6.MIR675, H1.MIR675, U6.MIR675-3p, and U6.MIR675-5p miRNAs. RenLuc-DUX4-FL contains DUX4 ORF and 3′UTR sequences fused to Renilla luciferase after the stop codon. Exons 1–3 are indicated (Ex1,2,3). *SD5 indicates silent mutation of a

cryptic splice donor in Renilla luciferase. Firefly luciferase is used as transfection control. b U6.MIR675 reduced relative Renilla luciferase activity in constructs containing full-length DUX4, DUX4 ORF only, or DUX4 3' UTR only (35 ± 3%, 34 ± 2%, and 24 ± 5%, respectively; P