

Cardiopulmonary Exercise Test to Quantify Enzyme Replacement Response in Pediatric Pompe Disease

Introduction: Enzyme replacement therapy (ERT) with Myozyme improved the prospect of Pompe disease patients. Our aim was to evaluate ERT acute effect on exercise capacity in pediatric Pompe patients.

Methods: Pompe patients (10-19 years) were evaluated before and two days after ERT using cardiopulmonary exercise testing (CPET), six minutes walking test (6MWT) and motor function test (GMFM-88).