Replagal Enzyme Replacement Therapy for Children With Fabry Disease

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The Hospital for Sick Children, Toronto, Ontario, Canada
Primary Objective(s):

- To assess the safety of Replagal at a dose of 0.2 mg/kg administered over 40 (+/-10) minutes in children with Fabry disease

- To assess the effect of Replagal on heart rate variability in patients 7 to 17 years of age

Secondary Objective(s):

- To determine the pharmacokinetics of Replagal at baseline and after the initiation of enzyme replacement therapy (ERT)

- To determine exploratory measurements of efficacy including renal function (ie, estimated glomerular filtration rate [eGFR] and creatinine clearance), clinical outcomes (in Cohorts 1 and 2), and sweating and left ventricular mass index (LVMI) (Cohort 1, Phase 1 only)