Method for treating glycogen storage disease

The present disclosure is directed to methods of treating a cytoplasmic glycogen storage disorder, including glycogen storage disease I, glycogen storage disease III, glycogen storage disease IV, and/or conditions associated with a PRKAG2 mutation, by administering a lysosomal enzyme such as acid alpha-glucosidase. Conditions associated with a PRKAG2 mutation may include hypotonia, cardiomyopathy, myopathy, cytoplasmic glycogen accumulation, ventricular hypertrophy, severe infantile hypertrophic cardiomyopathy, heart rhythm disturbances, increased left ventricular wall thickness, ventricular pre-excitation, or a combination thereof. Methods of treating a cytoplasmic glycogen storage disorder by administering a lysosomal enzyme and a second therapeutic agent are also described. Other embodiments are directed to methods of treating a cytoplasmic glycogen storage disorder by administering a therapeutic agent as an adjunctive therapy to lysosomal enzyme replacement therapy.

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