METHODS OF TREATING FABRY DISEASE IN PATIENTS HAVING A MUTATION IN THE GLA GENE

ABSTRACT

Provided are methods of treating a patient diagnosed with Fabry disease and methods of enhancing α-galactosidase A in a patient diagnosed with or suspected of having Fabry disease. Certain methods comprise administering to a patient a therapeutically effective dose of a pharmacological chaperone for α-galactosidase A, wherein the patient has a mutation in the nucleic acid sequence encoding α-galactosidase A. Also described are uses of pharmacological chaperones for the treatment of Fabry disease and compositions for use in the treatment of Fabry disease.

(FIG. 3)