Methods and Compositions for Delivering Enzymes and Nucleic Acid Molecules to Brain, Bone and Other Tissues

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Description:
Disclosed are methods for delivering an enzyme to a subject's brain or bone. The methods include administering a hyaluronidase to the subject and administering the enzyme to the subject. The hyaluronidase and the enzyme are administered to the subject under conditions effective to deliver the enzyme to the subject's brain or bone. Compositions and kits which include hyaluronidase and an enzyme are also disclosed, as are methods for increasing blood-brain barrier permeability in a subject. Also disclosed are methods, compositions, and kits for delivering genes or other nucleic acid molecules to a subject's brain or bone, as well as methods, compositions, and kits for delivering enzymes to a subject's tissues. The methods, compositions, and kits are disclosed as being useful in treating or preventing a variety of enzyme deficiency diseases, such as those affecting brain and/or bone, e.g., as Canavan's disease, Fabry disease, Gaucher's disease, various forms of mucopolysaccharidosis (e.g., Hurler's syndrome, Scheie syndrome, Hurler-Scheie syndrome, Sanfilippo A syndrome, Morquio A syndrome, Morquio B syndrome, etc.), Niemann-Pick disease, Schindler disease, and Pompe disease.

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