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AAV9 Gene Therapy for Type II GM1 Gangliosidosis

C.J. Lewis and Others

In nine patients with GM1 gangliosidosis, a fatal neurodegenerative disease, AAV9 β -galactosidase gene therapy was associated with short-lived adverse events, increased β -galactosidase levels, and delayed progression.

Change in LDL Cholesterol Levels

IMAGE CHALLENGE

What is the diagnosis?

ANSWER →

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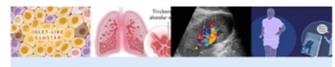
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