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Commentary

The Significance of Adeno-Associated Virus Gene Therapy

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DESCRIPTION

GM1 ganglionic is a rare, fatal neurological disorders caused by *GLB1* gene mutations and -intergalactic deficiency. Aden-Associated Virus (AAV) gene therapy treatment delays symptom onset and increases survival in a *GM1* ganglionic cat model, laying the groundwork for AAV gene therapy studies. The availability of verified biomarkers would considerably enhance therapy effectiveness evaluation. *GM1* gangliosidosis is a rare, fatal neurodegenerative genetic disease caused by mutations in the *GLB1* gene, which results in a lack of -galactosidase enzyme activity and the accumulation of glycoconjugates with a terminal -galactose, such as gangliosides *GM1* and *GA1*, oligosaccharides, glycopeptides, and keratin sulphate.

It is estimated that *GM1* gangliosidosis affects live births. Progressive motor and cognitive deterioration, visual abnormalities, and untimely mortality are the predominant clinical indicators. The illness affects systemic organs such as the liver, spleen, and skeletal structure in addition to neurological signs. The clinical manifestations of *GM1* gangliosidosis are diverse, and the disease is classified into three types based on age of onset: type I (infantile form), type II (late-infantile and juvenile forms), and type III (chronic or adult form), with disease progression and severity of symptoms increasing in the order type I>typeII>type III. Over variants in *GLB1* have been described, and there is no obvious relationship between genotype and phenotype. There are presently no effective treatments for *GM1* gangliosidosis, just symptomatic relief.

Adeno-Associated Viral (AAV) gene therapy that restores galactosidase activity is the most prominant medication in delaying symptom onset, reducing lysosomal storage in the brain and peripheral tissues, and increasing lifespan in the *GM1* gangliosidosis cat model, which extremely resembles late-infantile or juvenile *GM1* gangliosidosis. These remarkable discoveries laid the groundwork for the start of two AAV gene therapy clinical trials, and a third AAV-based clinical investigation is now ongoing. Because of the limited and varied patient group, as well as delayed advancement in non-infantile children, evaluating therapy success with clinical endpoints is difficult. As a result, using established biomarkers to anticipate therapeutic benefits is crucial in order to speed medication development.

Susceptibility/risk biomarker, diagnostic biomarker, monitoring biomarker, prognostic biomarker, predictive biomarker, pharmacodynamics biomarker, safety biomarker, and surrogate endpoints are the best-defined biomarker categories.

Biomarkers are used at various stages of drug development for a variety of purposes, including monitoring a therapy's safety with a safety biomarker, determining whether treatments produce needed results with a pharmacodynamics biomarker, predicting patients who may respond better to intervention with a predictive biomarker, and predicting clinical benefits more quickly than traditional clinical endpoints with surrogate endpoints.

While these biomarkers overlap with monitoring biomarkers, pharmacodynamics biomarkers that have been shown to correlate with more clinically significant endpoints might theoretically be produced as surrogate endpoints. Our objective is to find pharmacodynamics biomarkers in easily available human bio fluids including urine, plasma, and Cerebrospinal Fluid (CSF) to aid in the monitoring of gene therapy treatment effectiveness. Ideally, such biomarkers should have a wide dynamic range to distinguish between *GM1* gangliosidosis and control people, and they should return to normal after effective therapy.

We investigated the potential of ganglioside *GM1* and oligosaccharides as pharmacodynamics biomarkers for gene therapy in this work. Whereas ganglioside *GM1* was slowly increased in patient Cerebrospinal fluid and plasma, a Penta saccharide referred to as elevated more than 18-fold in patient CSF, plasma, and urine, as well as in the *GM1* gangliosidosis cats' Central Nervous System (CNS). We used mass spectrometry, chemical and enzymatic degradations to determine its structure.

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