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Adrenoleukodystrophy (ALD): from axonal degeneration to clinical trial readiness

Many neurodegenerative diseases are characterized by a progressive myelopathy. Currently, options to quantify myelopathy are limited. Quantifying disease severity is of the utmost importance in studies to determine efficacy of new treatments. Clinical trials to determine efficacy of new treatments for these disorders are challenging because: 1) disease progression is slow and 2) current outcome measures lack sensitivity to detect small changes in disease severity. Therefore trials require many participants and trial duration has to be long. New quantitative and sensitive outcome measures are needed. I recently demonstrated that optical coherence tomography (OCT) and quantitative MRI (DTI) distinguish between symptomatic and asymptomatic ALD patients. These techniques as well as force plate analysis are potential useful outcome measures. Finally, new techniques like lipidomics analysis might identify prognostic biomarkers to distinguish patients with slow and rapid disease progression.

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