

If you don't have a genetic confirmation for your Leigh's Syndrome (LS) diagnosis you could have Pyruvate Dehydrogenase Complex Deficiency (PDCD).

In one study of people with PDCD, 50 of 186 patients (27%) had Leigh's Syndrome based on neuroimaging studies. LS and PDCD share other similarities as well. The table below lists symptoms that are common to both diagnoses:

LS and PDCD Share Similar Phenotypes

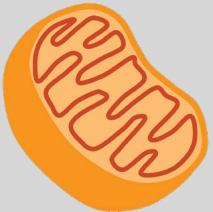
Symptom/Sign	LS ¹	PDCD ²
Develop. delay	X	X
Hypertonia/hypotonia	X	X
Seizures	X	X
Ataxia	X	X
Lactate (blood, CSF, brain)	X	X
MRI findings (esp. basal gang.)	X	X

1. Gerards M et al. Mol Gen Metal 117:300, 2016

2. Patel K et al. Mol Gen Metab 106:385, 2012

There is a Phase 3 Trial of Dichloroacetate (DCA) in Pyruvate Dehydrogenase Complex (PDC) Deficiency that is currently recruiting.

NOW RECRUITING
Clinical Trial:
DCA in PDC Deficiency



WHO? Patients with genetically confirmed pyruvate dehydrogenase complex (PDC) deficiency ages 6 months through 17 years.

WHERE? 9 academic health centers throughout the US. Visit <http://tinyurl.com/DCA-trial> to find a study site near you. Travel assistance is provided if needed.

WHY? This is a study of an investigational drug called sodium dichloroacetate (DCA). Investigational means that the study drug has not been approved by the US Food and Drug Administration (FDA) and that its use in this study is experimental. The purpose of this research study is to see if DCA improves health and function for patients with PDCD.

HOW? For more information contact Peter W. Stacpoole, PhD, MD, pws@ufl.edu