

Friedreich's Ataxia Treatment Update

The genetic cause of Friedreich's Ataxia (FA) was discovered in 1996, but only now is a new era of therapies for FA emerging.

The approval of omaveloxolone (SKYCLARYS™) by the US Food and Drug Administration (FDA) in February was a watershed moment. This is the [first disease-modifying drug](#) approved to treat FA.

“Overall, SKYCLARYS is a generally safe and beneficial treatment that will improve the lives of people with FA,” says David Lynch, MD, PhD, Neurologist, Children's Hospital of Philadelphia.

SKYCLARYS is a small-molecule drug that activates the Nrf2 transcription factor, inducing molecular pathways that promote the resolution of inflammation by restoring mitochondrial function, reducing oxidative stress, and inhibiting pro-inflammatory signaling. “The data from the [clinical trial](#) is that the initial treatment, on average, takes people back about a year and a half into the past in terms of their neurologic abilities,” Dr. Lynch says. “The disease begins to progress again two to three years after treatment, but that progression is still slower than natural history data for about four to five years after treatment.”

Dr. Lynch points to the [phase 2 trial](#) studying PTC743 (Vatiquinone) in children with FA as another area of promise. Vatiquinone is an oral small molecule designed to limit neuroinflammation and nerve cell damage in FA. Vatiquinone has been tested previously in adults with FA and was found to be safe and well-tolerated. Dr. Lynch expects data to be available this summer.

“Up to this point, there have been very minimal improvements in therapy, all of which are treating the symptoms of the disease, not the underlying path of physiology,” Dr. Lynch says. This year marks a critical step in the improvement of treatment for FA