

# First Medication to Treat Friedreich's Ataxia Approved by the FDA

Yesterday, the U.S. Food and Drug Administration (FDA) announced the approval of SKYCLARYS (omaveloxolone) manufactured by Reata Pharmaceuticals, Inc., Plano, TX 75024 USA for the treatment of Friedreich's Ataxia in adults and children 16 years of age and older ([FDA statement](#)).

[Friedreich's ataxia](#) (FA) is a genetic, progressive, neurodegenerative movement disorder caused by mitochondrial dysfunction affecting 1 in 50,000 people in the US.

SKYCLARYS is the first approved treatment for FA, a disease associated with mitochondrial dysfunction and an important step for all rare diseases where mitochondrial dysfunction plays a major part in the pathogenesis.

FA differs from the typical primary mitochondrial disease (PMD) in the fact that it is a monogenic disorder with a uniform phenotype caused by a common mechanism of action indirectly related to ATP production as opposed to the broader group of PMD which is genetically, phenotypically and mechanistically very heterogeneous.

This approval brings hope for our PMD. A drug to treat mitochondrial dysfunction regardless of its cause could hold the key for the treatment of genetically inherited mitochondrial diseases (PMD) and other more common disorders where mitochondrial dysfunction plays a major role (neurodegenerative conditions, cardiovascular disease and other metabolic conditions, etc.).

While the majority of Friedreich's ataxia patients will benefit from SKYCLARYS, it is not for everyone with FA or suffering from other diseases caused by mitochondrial dysfunction. Prescribing SKYCLARYS requires knowledge of a patient's medical history, as well as clinical monitoring for side effects and follow-up care to determine whether a patient is benefiting from it.

The Mitochondrial Medicine Society does NOT endorse the use of SKYCLARYS in the treatment of PMD at this time.

[A Phase 2 Study](#) of the Safety, Efficacy, and Pharmacodynamics of omaveloxolone in primary mitochondrial myopathy was conducted internationally in 53 adult subjects and failed to meet its primary end point in this population after a 12 week treatment (ClinicalTrials.gov Identifier: NCT02255422). The MMS and mitochondrial disease advocacy groups are constantly in discussions with industry partners like Reata and will be advocating for omaveloxolone to be re-evaluated in the PMD population.

We urge people with PMD to discuss questions they might have with their physician. If you don't have a mitochondrial disease expert, you may find one at one of our Mitochondrial Care Centers ([www.mitonetwork.org](http://www.mitonetwork.org)).

On behalf of the Mitochondrial Medicine Society (MMS) Board  
On behalf of the European Society of Mitochondria Medicine and Research (E-mit)



Omaveloxolone FA ClinicalTrials.gov Identifier: NCT02255435  
Omaveloxolone PMM ClinicalTrials.gov Identifier: NCT02255422

[To read the Friedreich's Ataxia Research Alliance \(FARA\) statement.](#)