

News Alert

September 14, 2023

Rocket Pharmaceuticals up 39% on good news for Danon patients

Aescap portfolio company Rocket Pharmaceuticals yesterday went up 39% after it came to an agreement with the FDA that it only needs to enroll 12 patients in a pivotal clinical trial with their gene therapy for a fatal heart disease. Final clinical studies usually involve hundreds to thousands of patients. Enrolling 12 people saves both time and costs. The FDA's decision follows excellent data from a phase 1/2 clinical trial for the treatment of Danon disease that showed the disease is not only halted but also reverted.

It's great to see that the FDA has a sense of urgency when it comes to supporting the development of medicines for severe diseases for which there are no treatments yet.

Rocket Pharmaceuticals

Other groundbreaking medicines from Rocket Pharma are two gene therapies for deadly blood diseases that already have been fully developed and are expected to be approved by the FDA next year. The company has a rapidly growing pipeline with other gene therapy treatments.



The Rocket Pharma offices, laboratories and manufacturing facilities(10.000 m2) are based in Cranbury in the state New Jersey

Rocket Pharma is a portfolio company in both Aescap Genetics (8%) and Aescap Life Sciences (1%). These percentages refer to the moment before the share price increase.

Danon disease

Most common symptoms of Danon disease are weakening of the heart and skeletal muscles. It is diagnosed during childhood up to early adulthood and leads to mortality in male patients at the average age of 20 and in females around 15 years later. There are currently 15.000 to 30.000 Danon patients in the U.S and Europe.