

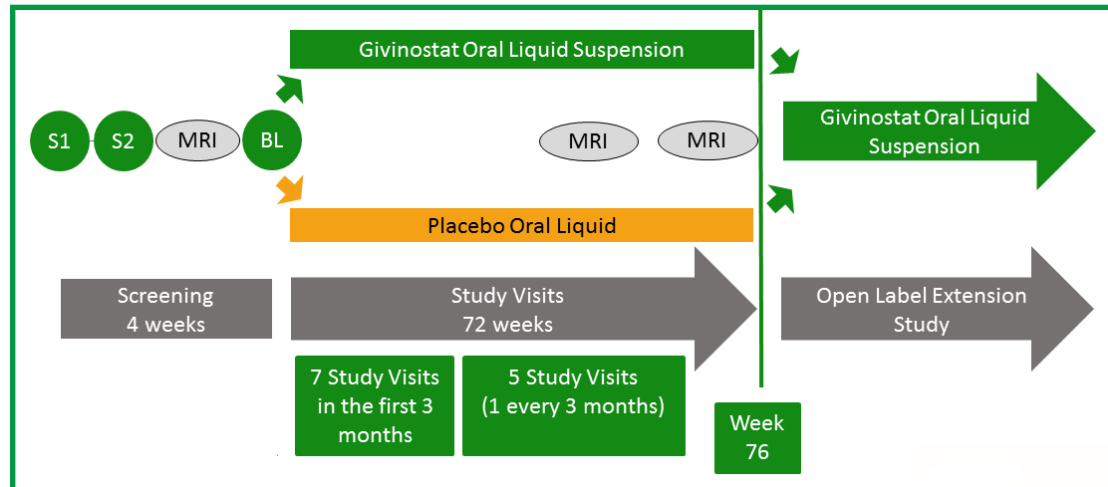


Community Newsletter

Issue 1 2021

Trial Update: Phase 3 DMD Trial Fully Enrolled

Clinicaltrials.gov #NCT02851797



The trial enrolled boys with DMD ages 6 and older who were walking, able to climb stairs, and met other study criteria. People with any genotype could participate. The purpose was to test whether givinostat affects the time to climb 4 stairs. Participants were randomly assigned to receive up to 72 weeks of givinostat or placebo. After the trial, everyone can enroll in an open label extension study.

An independent data monitoring committee completed a pre-planned interim analysis of the trial data. The committee determined the trial was not futile and that enrollment could continue. The trial met its enrollment goals in September 2020. A total of 179 participants enrolled at sites around the world. Study results are expected in the second quarter of 2022.

Givinostat in Becker Muscular Dystrophy (BMD)

We recently presented results for our Phase 2 clinical trial of givinostat in BMD (#NCT03238235). The trial enrolled 51 men with BMD ages 18 to 65 years living in Italy and the Netherlands. Participants were randomly assigned to take either givinostat or placebo for 12 months. The primary endpoint was change in total fibrosis in the muscle in muscle biopsy. Secondary endpoints were changes in fat fraction and contractile cross sectional area (CSA) of the muscle in MRI, functional test results, and strength test results. Givinostat did not show a statistically significant difference in total fibrosis compared with placebo. However, givinostat did show statistically significant differences in fat fraction and contractile CSA compared with placebo. People with BMD who took givinostat for 12 months showed less fat infiltration compared with those who took placebo. These results support further development of givinostat in BMD. Our scientists are continuing to analyze the data and to discuss next steps with regulatory agencies. We will update the community as we learn more.

Watch our presentation of these results here: <https://bit.ly/3a8Bqr8>

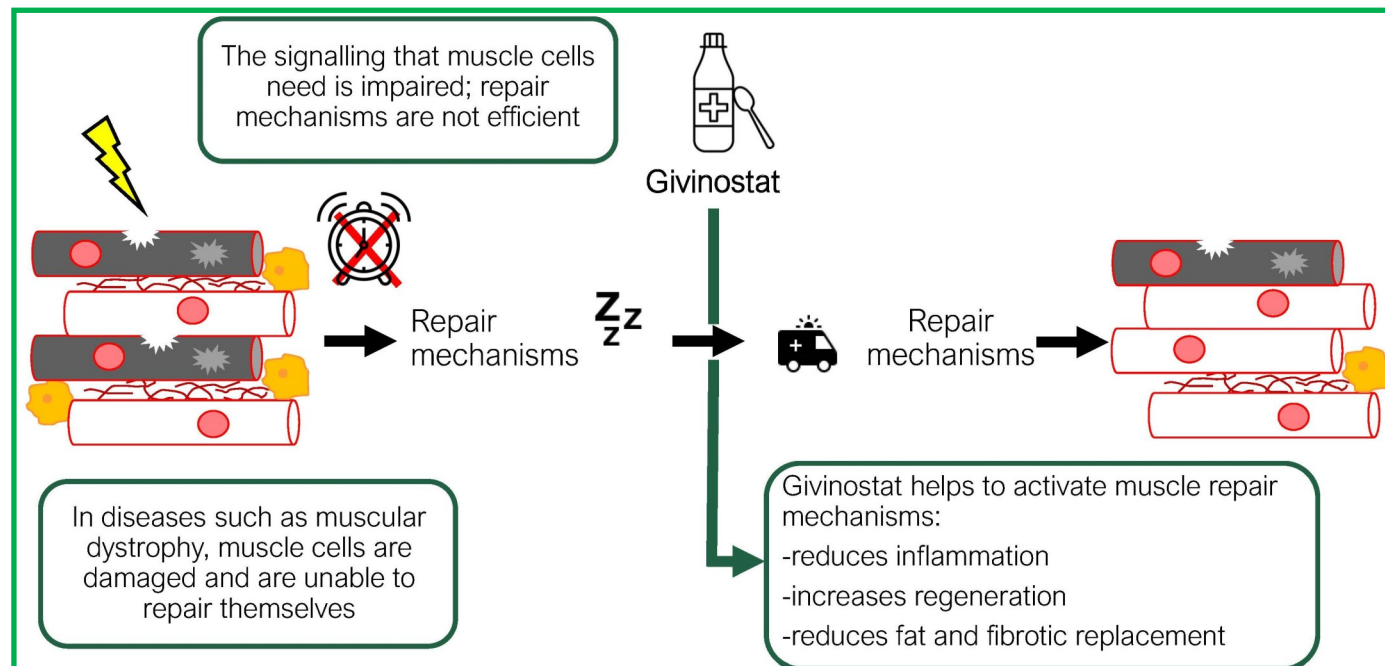
Would you like to learn more?

Email us: patientadvocacy@italfarmaco.com



ITALFARMACO

What is Givinostat?



Givinostat is an investigational drug. It acts as an inhibitor to allow the natural muscle repair process to work more normally. We are testing whether taking givinostat would improve muscle repair and maintenance for people with Duchenne muscular dystrophy (DMD).

People with DMD lack dystrophin. Dystrophin normally prevents cell damage. It also holds together a group of proteins which control muscle regeneration. Without them, the signals to start muscle repair don't work properly. Givinostat is a trigger to tell the cells to start repairs.

Watch our 2021 PPMD Conference presentation to learn more:

Frequently Asked Questions

I am concerned about SARS-CoV2 (COVID-19). What should I do?

Current study participants should ask the site study doctor or coordinator for help. Different strategies can help reduce the impact of local COVID-19 conditions on your study participation. Your trial site can best advise you on what you and your family should do.

What happens next? When will I hear more about the Italfarmaco program?

The Phase 3 DMD trial will have results available in 2022. Until then, we are planning to attend community conferences and workshops hosted by DMD/BMD patient advocacy groups. See you soon!

Thank You!

We are grateful for the support of the DMD and BMD community who are helping us to make our clinical trials a success. We would like to thank all the scientists, clinical trial investigators, trial site staff, patient advocacy groups, and all of the families who are hard at work to support this research.

We deeply appreciate everyone's commitment to learning more about investigational drugs for DMD.

Would you like to learn more?

Email us: patientadvocacy@italfarmaco.com

