



GFB

Gruppo Familiari Beta-sarcoglicanopatie Ets

Sede legale: Via Civasca 112, 23018 Talamona (SO) Italia
Cell. 3280075986, e-mail presidenza@beta-sarcoglicanopatie.it,
www.gfbonlus.it

NEWSLETTER N.1 - 2024

HAPPY EASTER



PHASE 3 OF EXPERIMENTAL GENE THERAPY (SRP-9003) AS A POSSIBLE TREATMENT FOR LGMD2E/R4 (EMERGENE) STARTED

This is a global multicentre study on the effects of SRP-9003 in participants with limb-girdle muscular dystrophy type 2E/R4 (LGMD2E/R4). This study will cover ambulatory (cohort 1) and non-ambulatory (cohort 2) participants. ambulatory (cohort 2), with a total of 15 people.

Currently the only centre open for the trial is Norfolk, Virginia - Children's Hospital of the King's Daughter.

More information about the study: <https://clinicaltrials.gov/study/NCT06246513?intr=emergene&rank=1>

or contact SareptAlly@sarepta.com or crystal.proud@chkd.org

WEBINAR ON SAREPTA'S EMERGENE STUDY

On Wednesday 10 April 2024 at 12:00 EST, a virtual community webinar on Sarepta's EMERGENE study (SRP-9003-301) has been organized. During this webinar a Sarepta speaker will provide an overview of the clinical development of SRP-9003, Sarepta's investigational gene therapy for LGMD2E/R4 (beta-sarcoglycanopathy).

Registration at the link: https://us02web.zoom.us/webinar/register/WN_D35A0cKIR3aynzsW7XQqgg?fbclid=IwAR3BCi00DL91I2rKvIkqVk8pXJlbaO8L3Isuo12WF_ylobMX79zSm5KY18U#/registration

LIVE
COMMUNITY WEBINAR

EMERGENE Study (SRP-9003, Study 301)

The Speak Foundation is Excited to Host Sarepta this Spring!

Sarepta will be giving a presentation to the community to provide a study update on the LGMDR4/2E program. This is an opportunity to hear directly from Sarepta and engage in a live Q&A on their EMERGENE study.

LGMD R4/2E | April 10 2024 | Begins 12 PM EST

Scan the QR Code to Register Today!

Questions? ContactUs@TheSpeakFoundation.com • Advocacy@Sarepta.com

You do not have to have LGMDR4/2E to register and attend. This webinar is open to ALL members of the LGMD community.

NATURAL HISTORY STUDY FOR LGMD2A

On Wednesday 3 April 2024 at 1pm EDT the Coalition to Cure Calpain 3 has organised a webinar where Sarepta will talk about the Natural History Study for LGMD2A.

More information at the link: https://us02web.zoom.us/webinar/register/WN_D35A0cKIR3aynzsW7XQqgg?fbclid=IwAR3BCi00DL91I2rKvIkqVk8pXJlbaO8L3Isuo12WF_ylobMX79zSm5KY18U#/registration

COALITION TO CURE CALPAIN 3
IS PLEASED TO WELCOME
SAREPTA TO PRESENT
"JOURNEY UPDATE FOR THE
LGMD2A/R1 CALPAINOPATHY COMMUNITY"
a new natural history study for
individuals living with LGMD2A/R1

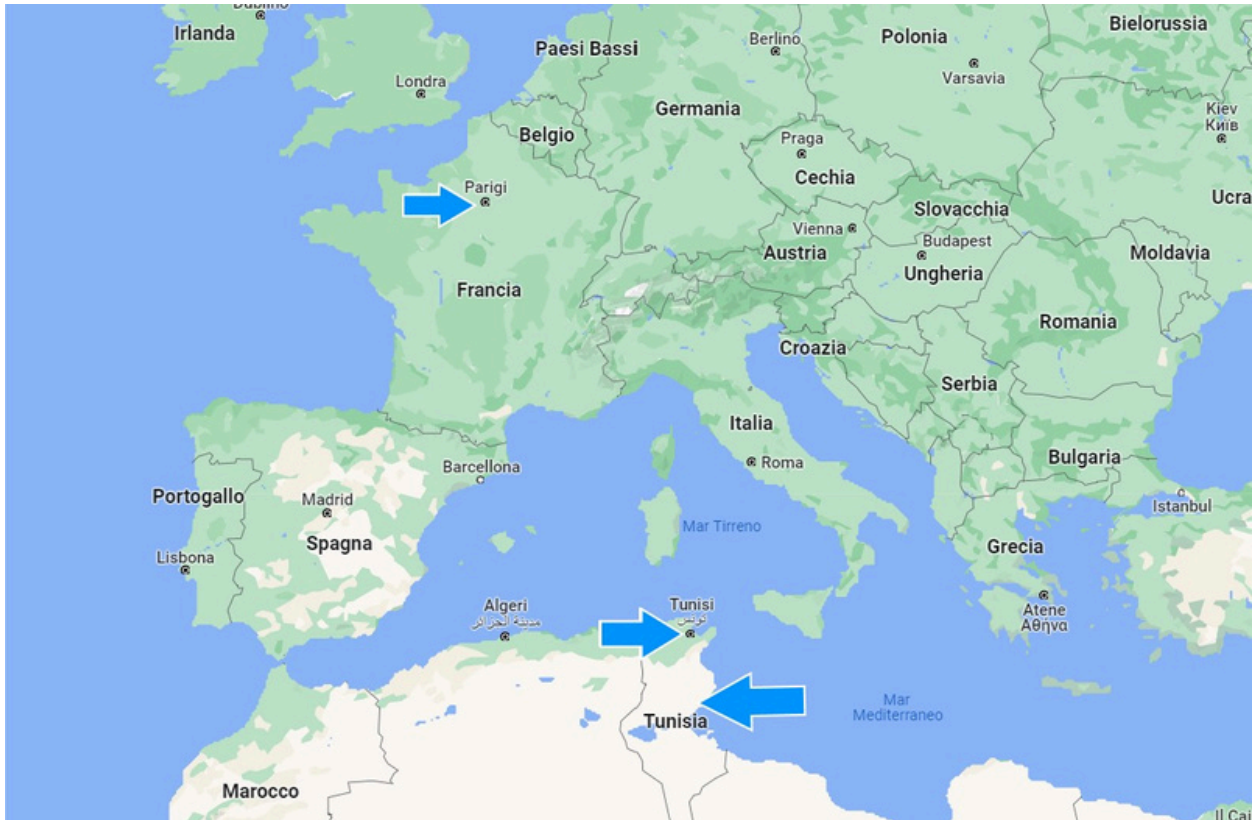
JOIN US FOR A "LUNCH BREAK" WEBINAR
WEDNESDAY, APRIL 3
NOON - 1 PM EDT

REGISTER AT THE LINK IN THE
COMMENTS SECTION TO TUNE IN LIVE
After registering, you will receive a confirmation email
containing information about joining the webinar.

THIS EVENT WILL BE RECORDED

NATURAL HISTORY STUDY FOR LGMD2C-R5

Atamyo Therapeutics has published on clinicaltrials.gov its clinical study for LGMD2C-R5, on 50 patients, aged between 6 and 35 years, divided into two groups. Three clinical centres will be involved, one in France and two in Tunisia. Full details of the study can be found at this link: <https://classic.clinicaltrials.gov/ct2/show/NCT06210672?term=atamyo&cond=lgmd2c&draw=2&rank=2>



GENE THERAPY STUDY FOR LGMD2C-R5

The gene therapy study for LGMD2C-R5 has been published on clinicaltrials.gov. The clinical centres involved will be in Paris, France and Milan, Italy. More details of the study at this link: <https://classic.clinicaltrials.gov/ct2/show/NCT06210672?term=atamyo&cond=lgmd2c&draw=2&rank=2>

QUALITY PROJECT UPDATES

GFB's Quality project will continue with the completion of questionnaires until 31 December. At the moment, more than 600 questionnaires have been completed. In 2025, they will then be analysed statistically. Recruitment is still open. To participate, please write to segreteria@beta-sarcoglicanopatie.it. For further information: <https://www.beta-sarcoglicanopathy.org/?view=article&id=227&catid=2>



UPCOMING GFB ACTIVITIES

13 April 2024 | GFB will attend GMN - Neuromuscular Disease Day 2024 in Milan with Bianchi Lucica

22-25 April 2024 | GFB will attend the Myology 2024 congress in Paris with Prof. Yvan Torrente, Dr. Sanchez Carles Riera and Dr. Chiara Villa

5-8 June 2024 | GFB with Carles Sanchez Riera will attend the AIM 2024 congress in Rome

25-29 October 2024 | GFB will attend the ICNMD 2024 congress in Perth - Australia