GFB
Gruppo Familiari Beta-sarcoglicanopatie
Sede legale: Via Civasca 112, 23018 Talamona (SO) Italia
Cell. 3280075986, email info@beta-sarcoglicanopatie.it, www.gfbonlus.it

NEWSLETTER N.2

GFB SCIENTIFIC WEBINARS | MARCH 24, 2023

A new GFB scientific webinar will be held on March 24 at 3 pm. The webinar will be held by Dr. Simone Spuler (MD, Charite, University Medicine-Berlin, Germany) who will talk about cell therapy for sarcoglycanopathies: a clinical study of Alpha-sarcoglycan.
Registration is available at this link:
https://us02web.zoom.us/webinar/register/WN_UbuxPL1ERriDf46IU9ADGg

SAREPTA THERAPEUTICS ANNOUNCED THE START OF TWO CLINICAL TRIALS FOR LGMD2E AND LGMD2B

Sarepta is pleased to present two clinical trials in the area of developmental work in cingulate muscular dystrophy (LGMD): The VOYAGENE study for LGMD2E/R4 and the NAVIGENE study for LGMD2B/R2.

VOYAGENE (SRP-9003-102) for LGMD2E/R4: Screening is open for the VOYAGENE study, a multicenter, open-label, single-dose systemic gene transfer study in the United States to evaluate the safety, tolerability, and efficacy of SRP-9003 in individuals with LGMD2E/R4 (β-sarcoglycanopathy). VOYAGENE will enroll approximately five individuals and will include ambulant and nonambulant subjects. Further details can be found in the SRP-9003 section of this letter.

NAVIGENE (SRP-6004-102) for LGMD2B/R2: In the first half of 2023, Sarepta plans to initiate a Phase I proof-of-concept study called NAVIGENE (SRP-6004-102) to evaluate systemic administration in individuals with LGMD 2B/R2 (dysferlinopathy). They plan to enroll approximately 2 ambulatory individuals.

https://www.sarepta.com/community-letter-community-update-lgmd-programs-development

SAREPTA THERAPEUTICS ANNOUNCES THE START OF VOYAGENE, A CLINICAL TRIAL ON SRP-9003 FOR THE TREATMENT OF LGMD2E/R4

Sarepta Therapeutics announces the initiation of VOYAGENE, a clinical trial of SRP-9003 for the treatment of muscular dystrophy type 2E/R4.
Sarepta Therapeutics, Inc. announced that the first patient in the SRP-9003-102 study has been administered.

Also known as VOYAGENE, study 9003-102 is a phase 1 study of SRP-9003 (bidridistrogene xeboparvovec) for the treatment of muscular dystrophy type 2E/R4 (LGMD2E). VOYAGENE is a U.S.-only study enrolling ambulatory patients aged 18 years and older and nonambulatory patients aged 4-50 years using SRP-9003 clinical trial material.
Read the full article at this link: https://investorrelations.sarepta.com/news-releases/news-release-details/sarepta-therapeutics-announces-initiation-voyagene-clinical

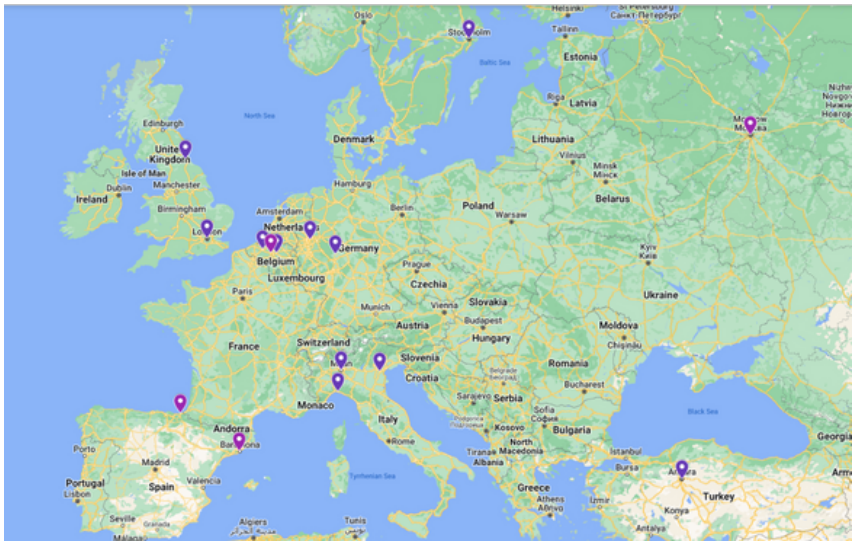
SPAIN ALSO JOINS THE JOURNEY PROJECT

Sarepta Therapeutics (Massachusetts, USA) JOURNEY PROJECT Update: A natural history study for patients with LGMDR3/2D, LGMDR4/2E and LGMDR5/2C.
The Journey study has been expanded to 2 additional centers in Europe and the USA:

SPAIN
Hospital Sant Joan de Déu Universidad de Barcelona
Barcelona, Spain, 08950
Contact SareptAlly@sarepta.com
Principal Investigator: Ortez Gonzalez, MD

OREGON, USA
Oregon Health and Science University
Portland, Oregon, United States, 97068
Contact SareptAlly@sarepta.com
Principal Investigator: Erika Finanger, MD

For information contact: SareptAlly@sarepta.com
Web site: https://clinicaltrials.sarepta.com/journeyLGMD



2013-2023 | 10 YEARS OF GFB

GFB CELEBRATES ITS FIRST 10 YEARS OF RESEARCH: 2013-2023. CELEBRATE THIS IMPORTANT MILESTONE WITH US TOO !

GFB Onlus' history begins back in 2013 when it represented people with LGMD, in front of institutions, research organizations, other associations, and patients. It has promoted, supported, and funded scientific studies aimed at targeted therapeutic approaches for this disease. It has developed adequate natural history studies, studies that are vital to advance research to help find a treatment and cure for LGMD.
https://www.beta-sarcoglicanopathy.org/scientific-research.html

GFB has organized training courses to improve personal care, designed by youth/young adults with motor disabilities and born out of their need to feel "cared for" and "understood". Many projects and initiatives have been planned: including the creation of a patient registry for Sarcoglycanopathies, sending newsletters in Italian, English and German, organizing mountain trips, sports for people with disabilities or recreational activities, psychological support for disabled people and their families, and courses for educators and volunteers.
https://www.beta-sarcoglicanopathy.org/gfb-odv/what-we-do.html

This and much more we will continue to do! Together for research!

MAKE THE HISTORY OF LIMB-GIRDLE MUSCULAR DYSTROPHIES WITH US

RARE DISEASE DAY | FEBRUARY 28, 2023

This year we celebrated the 16th Rare Disease Day, an important "birthday" that comes just days after the approval of a new equity-promoting tool: The Final Text of the National Rare Disease Plan. As required by the Consolidated Text, the approval process for the NRP will now continue with passage through the State-Regions Conference. The Plan is the result of a lot of work by all the institutions, clinicians, patient associations and those with expertise in rare diseases that make up the CoNaMr (National Committee on Rare Diseases).

Like every year, GFB Onlus also participated in the Day to raise awareness toward people with rare diseases. In addition to the importance of scientific research, which GFB Onlus has been involved in for 10 years, it is important to raise awareness of the impact that these diseases also have on the family context: as uniamomalattierare reminds us, 8 out of 10 have difficulty managing the more "ordinary" aspects of the affected person's and family's lives.

Through its activities, GFB Onlus wants to raise public, institutional and political awareness of the need for Research, simplification, early diagnosis and the needs of those who live with a rare disease every day.

On the occasion of this day, through all communication channels and social media, GFB Onlus promotes and shares the initiatives, online and in-person, organized around the world by GFB patients, creating a network between patients, families, caregivers and health and research professionals.

RARE DISEASE DAY®

XXI TELETHON SCIENTIFIC CONVENTION

GFB will participate in the XXI Scientific Convention organized by Telethon to be held March 13-15, 2023 in Riva del Garda (TN), Italy. More information at the link: https://www.telethon.it/cosa-facciamo/supporto-alla-ricerca-e-ai-pazienti/supporto-alla-ricerca/convention/la-convention-scientifica/

GFB UPCOMING EVENTS

MARCH 10-11 | GFB will participate in the National Congress of Pediatric Neurology to be held in Tunis where an abstract and poster will be presented with the latest updates on the Quality Project.

MARCH 13-15 | GFB will participate in the XXI Scientific Convention organized by Telethon to be held in Riva del Garda (TN).

MARCH 18 | GFB will participate in the Neuromuscular Disease Day will be held in attendance on March 18, 2023 in 18 Italian cities. To read the program for each city and register https://www.giornatamalattieneuromuscolari.it/

JUNE 8-10 | GFB will participate in the AIM Congress - Padua, Italy