



GFB

Gruppo Familiari Beta-sarcoglicanopatie

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QUALITY PROJECT

QUALITY PROJECT UPDATES

The QUALITY project is sponsored by the GFB organization to structure international patient data collected in past years, study patients' quality of life, and encourage patients' participation in natural history studies and clinical trials.

In December, the 4th phase of the Quality project began. In the first 3 phases, 327 questionnaires were completed. There are 165 patients participating in the project, suffering from Lgmd2c/R5, LGMD2e/R4 and Lgmd2d/R3.

Recruitment is still open by writing to segreteria@beta-sarcoglicanopatie.it.

The project's growth and excellent results are due to the extraordinary work of GFB Staff Interpreters, GFB's Russian and Arabic language groups, ADM Argentina Association and NMI Iran.

For more information visit www.lgmd2e.org



GFB'S NEW GROUPS

New staff joined GFB in 2022 with the aim of creating language groups that could follow and support patients living abroad.

In addition to the existing Iran group coordinated by Dr. Seyed Jafer Seyed, Irina Derevenets, GFB's Russian interpreter who coordinates the group for patients living in Russia and Ukraine, joined GFB.

Raja Younsi is the Arabic interpreter coordinating the group from Arab countries from Morocco to the UAE.

More information can be found on GFB's website at the following link: <https://www.beta-sarcoglicanopathy.org/gfb-odv/268-international-groups.html>

GFB SCIENTIFIC WEBINARS

A new webinar coordinated by Dr. Dorianna Sandonà is scheduled for Friday, February 10, 2023, during which she will discuss CTFR Correctors in Sarcoglycanopathies: a possible drug therapy. To participate, one must register at www.lgmd2e.org.

The webinar will be held in English but Italian translation will be available on the webinar chat, French and Russian translations on GFB whatsapp groups.

The recording will be available on the [GFB Onlus Youtube channel](https://www.youtube.com/channel/UCqBnLUS).



In 2022 GFB organized three different webinars funded by Sarepta Therapeutics. The webinars were held in English, but Italian subtitles can be activated on Youtube.

- Webinar on the JOURNEY natural history study held by Prof. Yvan Torrente
- Webinar on patient registries for LGMDs and updates on gene therapy projects held by Prof. Volker Straub
- Webinar on gene therapy projects developed by Atamyo Therapeutics held by Prof. Isabelle Richard



2022 CONGRESS

In 2022, GFB participated in international congresses on muscular dystrophies where it presented an abstract and posters with updates on the Quality Project and the study on patients' diagnosis that is still ongoing.

- July 5-7, 2022 : ICNMD 2022 in Brussels, Belgium
- September 12-15, 2022 : Myology 2022 in Nice (France)
- October 11-15, 2022 : World muscle society 2022 in Halifax (Canada)
- October 19-22, 2022 : AIM National Congress in Matera (Italy).

Oral presentations by GFB contributors at congresses can be found on GFB's Youtube page:
<https://www.youtube.com/playlistlist=PLKM8eCjMkclQn6ZOVJM4138Vkf11rKRnt>

NATIONAL CONGRESS OF PEDIATRIC NEUROLOGY | TUNISIA

GFB will participate in the National Congress of Pediatric Neurology to be held in Tunisi on March 10-11, 2023. GFB will present an abstract and poster with the latest updates on the Quality Project



INTERNATIONAL CONFERENCE ON MUSCULAR DYSTROPHIES

From October 27-29, 2023, the International Conference on Muscular Dystrophies will take place in Washington, United States organized by The Speak Foundation. The event will be in-person and registration is open from April 5, 2023.



RARE DISEASE DAY | FEBRUARY 28, 2023

Rare Disease Day will be held on February 28 as it is every year. GFB is organizing activities and events to raise awareness and improve access to care and medical representation for people with rare diseases and their families.



NEUROMUSCULAR DISEASE DAY | MARCH 18, 2023

Neuromuscular Disease Day will be held on March 18, 2023 in 18 Italian cities. The events will be in attendance. To read each city's program and register, visit the website: <https://www.giornatamalattieneuromuscolari.it/>



UPDATES ON THE LIFE PROJECT

In 2022 GFB participated in meetings organized by CSV (Volunteer service center) to create "Life Projects" in the Valtellina territory. The Project of Life is an individual project for each individual "person with physical, mental and/or sensory disabilities, stabilized or progressive," through which to create personalized paths in which the various interventions are coordinated in a targeted manner, thus maximizing the beneficial effects of the same and succeeding in responding to the needs of the beneficiary.

As of 2023, CSV has defined with Institutions and Entities a working model for collecting and processing data that the person brings, for the Multidimensional Assessment, for the construction of a Project Budget and the monitoring of its implementation. It also periodically organizes a Table for Life Projects chaired by ATS (health care office) on the work progress of the Life Projects planned for each Area.

UPDATES ON NATURAL HISTORY STUDY JOURNEY

The Natural History study Journey has been expanded to 12 other clinical centers, 5 in the US and 7 in Europe:

EUROPE

Belgium, Ghent

Belgium , Leuven

Sweden , Stockholm

Germany, Essen

United Kingdom, London

Italy, Genoa

Italy, Padua

UNITED STATES.

California, San Diego

California, Sacramento

Pennsylvania, Philadelphia

Texas, Dallas

Utah, Salt Lake City

For information contact: SareptAlly@sarepta.com

Web site: <https://clinicaltrials.sarepta.com/journeyLGMD>

