Newsletter n.3 - 2024

Save Ahmed and his family from Gaza

(In the picture in the middle Ahmed and his father and on the sides the Caritas volunteers who brought the food package)

The GFB Foundation organised an ‘Escape from Gaza’ fundraiser in May to help Ahmed and his family escape from Gaza to Egypt.

At the moment a total of $8994.51 has been donated and the foundation has already sent the family two payments of $25,000 and $2,000.

At the following link on the GFB website you can find all the updates on Ahmed's story

Read more

Quality Project Updates

The GFB Quality Project continues with the completion of questionnaires until 31 December 2024. A total of 270 people are registered with GFB

PARTICIPANT STATISTICS

At the moment 206 people from 44 countries have participated in the Quality Project:

65 with LGMD2D/R3 88 with LGMD2E/R4 53 with LGMD2C/R5.

683 questionnaires were completed (224 for 2D/R3, 173 LGMD2C/R5, 286 LGMD2E/R4)

LANGUAGE GROUP STATISTICS

27 participants from the Arabic group

35 participants from the Iran group

20 participants from Russian group

5 participants from Indian group

19 participants from the Spanish language group

PROJECT CONCLUSIONS

24 people have already completed the 7 stages of the questionnaire.

All participants must complete the last questionnaire by the end of December, which will give us a final picture of the clinical situation.

For further information please write to segreteria@beta-sarcoglicanopatie.it

DATA TRANSFER

We are still working on transferring the data to the Treat-nmd Global TGDOC Registry in the UK as soon as possible.

The GFB Quality Project continues with the compilation of questionnaires until 31 December 2024, when it will end.

First gene therapy for Duchenne approved in the US

Sarepta Therapeutics announced the approval by the Food and Drug Administration (FDA) in the US of an expansion for ELEVIDYS (delandistrogene moxeparvovec-rokl) to include individuals of at least four years old with Duchenne muscular dystrophy (DMD) and with a confirmed mutation.

Read more

Upcoming GFB activities

25 July - 1 December 2024 | Fourth charity raffle ‘You donate .... they get cured’

25-29 October 2024 | GFB will participate in the ICNMD 2024 congress in Perth - Australia with two scientific posters

8-10 November 2024 | Dr. Sanchez Carles Riera will attend the ENMC Workshop - The Netherlands

6-8 February 2025 GFB will present 2 scientific posters at the Treat-nmd conference in Dubai

17 and 18 March 2025 | GFB will participate in the 8th Conference of Networked Associations during the Telethon Foundation Scientific Convention

18-21 July 2025 | GFB will present at the International LGMD Conference - Orlando USA with

two scientific posters

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