



GFB

Beta-Sarcoglycanopathy Family Group

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Extraordinary Newsletter - September 2016

GFB ONLUS HAS SENT OTHER 200.000 \$ TO COLUMBUS

Another big effort for the association with its offices in Talamona in the province of Sondrio. On Friday, 9th September GFB ONLUS sent another contribution to Columbus Ohio, of **202.500 \$**. From 2012 up to now we have financially supported the American laboratories with a total of 1.300.000 \$. This further contribution will permit Prof. Mendell to go on with the project of GENE THERAPY FOR LGMD2E. GFB is at the moment the only financier of the project and without this support the project is destined to stop. The funds sent will be necessary for preparing the systemic clinical trial in high doses, that should be carried out in 2017.

This amount now sent will not be sufficient to make the clinical trial re-start. It is foreseen a further bank transfer of the association to Columbus within the end of the year.

You will find all the info about the project to the following link :

http://www.beta-sarcoglicanopatie.it/index.php?option=com_content&view=article&id=166&Itemid=111



GENE THERAPY IS GOING TO BECOME A DRUG

The gene therapy injected in patients affected by SMA1 has given very good improvements in the first 15 children treated in Columbus Ohio. Thanks to these results it will be possible to develop an abbreviated procedure to make this therapy become a drug. This procedure can be used since the therapy has been developed in order to treat a serious disease that is dangerous for the life and permits an important improvement.

This makes us hope also for the use of this therapy for our LGMD2E.

To this link it is possible to read the article, published on the website of the American Association Cure Sma:
<http://www.curesma.org/news/avexis-receives-breakthrough.html?referrer=https://www.facebook.com/>

HELP US TO FINANCE THE THERAPY

GFB ONLUS intends to continue also in the future to support this project in Columbus. For supporting the project of gene therapy for LGMD2E, GFB ONLUS has created a special FUND FOR RESEARCH through its current account of Banca Prossima. All the donations received on that account will be used to support the American projects and the scientific research on LGMD2E. Be one of us, and give your support with a donation on the bank account:

HEADING FONDO PER LA RICERCA: Gruppo Familiari Beta-sarcoglicanopatie Onlus

IBAN: IT33X0335901600100000076500 BIC /SWIFT code BCITITMX

GFB ONLUS ASKS FOR SUPPORT TO BANKS

THE FUNDS COLLECTED IN THESE YEARS ARE NOT SUFFICIENT TO SUPPORT THE PROJECTS OF THE ASSOCIATION, therefore starting from now GFB ONLUS is addressing to banks and is getting some loans.

The first loan already requested is "SUBITO 5X1000", through which our association has already received a contribution of 5x1000 of this year of 8.048,46 €.

Moreover we are informing for getting other loans, among which "Terzo Valore", whose news will be given in the next newsletters.

OTHER PATIENTS AFFECTED FROM LGMD2C IN GFB ONLUS

In the last months GFB ONLUS has found 5 other patients affected from Lgmd2c-2d-2e. These patients are from Tunisia, the Philippines, United States and Italy (Pescara). GFB counts now a total of 126 patients affected from Sarcoglycanopathy, so divided:

	LGMD2C	LGMD2D	LGMD2E	LGMD2F
2010	0	1	5	0
2013	4	15	14	1
2014	9	28	21	1
2015	12	54	28	1
2016	22	69	34	1

On the website of the association you can find the list of the patients sorted by geographical provenance to the link :

http://www.beta-sarcoglicanopatie.it/index.php?option=com_content&view=article&id=46&Itemid=54
