GFB

Beta-Sarcoglycanopathy Family Group

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Newsletter no. 1 - July 2017

GFB ONLUS STARTS DREAMING AGAIN,

NOW MUSCULAR DYSTROPHY HAS THE COUNTED DAYS:

Within 2027 there will be five new therapies

Graet emotion at GFB Onlus, that has reached an important achievement in scientific research for curing the limb-girdle muscular dystrophy, a rare form of dystrophy that in a short time does not permit to walk anymore, then to breath and then it stops the heart.

On June 15th our Onlus **signed an important agreement with an American Company - Myonexus Therapeutics –** with the objective of achieving, within 2027, the acknowledgment of five new specific therapies for different forms of limb-girdle muscular dystrophies.

With this agreements GFB Onlus has officially been admitted to the said Company and **HAS SENT TO COLUMBUS OTHER 200.000 EUROS** for supporting the project.

Starting from 2012 this project- carried out by Professor Jerry Mendell at the Nationwide Children's Hospital of Columbus, Ohio, in the United States – **has exclusively been financed by GFB Onlus,** with a total amount of 1.322.500 dollars.

The results obtained in these years are in two scientific publications and the pre-clinical phase has been completed thanks to the great commitment of GFB Onlus, only financing body. «Just last year, but, GFB Onlus was afraid not to be able to continue alone with the development of this therapy – says Beatrice Vola, chairman of GFB Onlus – the future appeared to be uncertain … but this year there was an important change and after a year we had to stop because of the lack of funds, finally now the American project of genetic therapy can restart». It begins so a "new era" for GFB Onlus, that is not alone anymore to finance this project: other groups of American families have joined the Company. They have a similar story to GFB, to underline that what is in Italy it is also in other countries, where the resources seem to be more. These families' children suffer from an unknown illness, there are no bodies financing the researches and the families feel to be forgotten. So they decide to create foundations on their own, that operate in this field.

Now these foundations are joining to GFB Onlus in the Company Myonexus and all together they be stronger!

The **Company has been active in founding more and more funders** and already an important American company has been involved, this is REV1 Ventures.

Moreover other bodies are getting in touch with the Executive Director of the company, Dottor Michael Triplett. For further info see link www.myonexustx.com .

" the programme of the company is very ambitious, **within 2027 there will be five new therapies** for some forms of limb-girdle muscular dystrophies (LGMD2B-2C-2D-2E-2L) – says Mrs Vola enthusiastic – This year the trial on patients with 2E will start, and that possible thanks to the project financed by GFB ONLUS, to pass then in the next years to the other four diseases. There will be injected the first 6 patients by systemic route, at dosages much higher.

The therapy will reach the whole body, heart too. In 2017 almost 2.000.000 dollars will be spent on trial with 2E, that will involve much more patients".

GFB is now working actively to find other financiers for the Company and to reach another important goal: **to take the therapy also to Milan in few years – in 2020**. With these goals, GFB Onlus has started the campaign "Let's treat them 4.0" for improving patients' quality of life suffering from limb-girdle muscular dystrophy.

«All this seems to be still a dream for the members of the association – says Beatrice Vola – but it was possible thanks to the many supporters, in particular to all those that participated in the loan "Terzo Valore" and to all those that supported the first campaign SMS of GFB in May.

Thanks to the collected funds we now think to be already able to send a further transfer to the Company next year.

The association thanks its friends Luca Ciaponi, the families of Roma, Ancona, Viareggio, Lecco, Como, Sondrio, a French family and the bodies that participated, Cooperativa Orizzonte, Proloco of Paniga, group Presepe Cà Giovanni, the theatre company Amici Anziani, Tecnici Senza Barriere, our friends skiers, Nuovo Pignone of Talamona, the association Amici del Bambino, the sections Uildm of Sondrio and Lecco, the working group Association Amici Anziani ».

All further info at www.lgmd2e.org

PUBLISHED THE SECOND PHASE OF THE AMERICAN PROJECT OF GFB ONLUS

On April 5th the magazine Molecular Theraphy published the article "Systemic AAV-Mediate -Sarcoglycan Delivery Targeting Cardiac and Skeletal Muscle Ameliorates Histological and Functional Deficits in LGMD2E Mice". In the article are all the results of the second part of the pre-clinical phase of the project of of gene therapy financed by GFB ONLUS.

See the article at the link: https://www.ncbi.nlm.nih.gov/pubmed/28284983

OTHER PATIENTS AFFECTED FROM LGMD2C-2D-2E IN GFB ONLUS

In the last months GFB ONLUS has found other patients affected from Lgmd2c-2d-2e. GFB counts now a total of 203 patients affected from Sarcoglycanopathy, so divided:

	LGMD2C	LGMD2D	LGMD2E	LGMD2F	SARCOGL.
2010	0	1	5	0	
2013	4	15	14	1	
2014	9	28	21	1	
2015	12	54	28	1	
2016	23	77	70	1	3
2017	28	85	86	1	3

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

GFB ONLUS HAS PARTICIPATED IN ENMC WORKSHOP

In March Prof. Yvan Torrente represented GFB ONLUS at the Workshop organised by ENMC (European Neuro Muscular Centre), held in Narden, in Netherlands. Title of the workshop “Limb Girdle Muscular Dystrophies, Nomenclature and reformed Classification”. During the workshop it was introduced a new nomenclature for the Limb Girdle Muscular Dystrophies.

http://www.enmc.org/publications/workshop-reports/limb-girdle-muscular-dystrophies-nomenclature-and-reformed-classification

GFB ONLUS HAS PARTICIPATED IN THE CONVENTION TELETHON

Dr. Carles Sanchez Riera, member of the Medical and Scientific Commission represented GFB ONLUS at the Convention Telethon, held in Riva del Garda, Italy, in March. He participated at the convention of the Associazioni Amiche of Telethon and at the meeting of Coordinamento Associazioni Malattie Neuromuscolari CAMN (Coordination Associations for rare neuromuscular diseases)

GFB ONLUS HAS RECEIVED OTHER CONTRIBUTIONS

The GFB Onlus has received the following contributions:

2017:

__82.435	Quote prestito Terzo Valore, Proloco di Paniga, Tecnici Senza Barriere, sezione UILDM di Lecco, Amici Sciatori e 27privati
51.565 €	Donazioni Terzo Valore, amici di Luca Ciaponi, Cooperativa Orizzonte, compagnia teatrale Amici Anziani, gruppo Presepe Cà Giovanni, associazione Amici del Bambino, sezione Uildm di Sondrio e 34 privati
65.600 €	Prestito Banca Prossima
18.526 €	Biglietti lotteria GFB

225 €	Tessere sostenitori GFB
9.872 €	Contributo 5x1000 relativo al 2015
50 €	Donazione NN per la ricerca scientifica
100 €	Donazione NN per la ricerca scientifica
20 €	Donazione NN per la ricerca scientifica
150 €	Donazione Coscritti 1941 di Talamona
30 €	Donazione NN per la ricerca scientifica
50 €	Donazione NN per la ricerca scientifica
100 €	Donazione NN per la ricerca scientifica
1.200 €	Donazione NN per la ricerca scientifica dalla Francia
100 €	Donazione NN per la ricerca scientifica
500 €	Donazione NN per la ricerca scientifica
200 €	Coscritti 1949 di Talamona in memoria di Vito
500 €	Gruppo di lavoro Associazione Amici Anziani Talamona
1.300 €	Donazione Nuovo Pignone GE
5.973,09 €	Bando Home Care Premium
20 €	Donazione NN per la ricerca scientifica
250 €	Donazione NN per la ricerca scientifica
11.530 €	Convenzioni con Enti Pubblici
500 €	Donazione NN per la ricerca scientifica
20 €	Donazione NN per la ricerca scientifica
50 €	Donazione NN per la ricerca scientifica
6.738 €	Campagna SMS maggio 2017 qui la campagna SMS
50 €	Donazione NN per la ricerca scientifica
15 €	Donazione NN per la ricerca scientifica
10 €	Donazione NN per la ricerca scientifica
879 €	Donazione Torneo Cral Nuovo Pignone per la ricerca scientifica
50 €	Donazione NN per la ricerca scientifica
20 €	Donazione NN per la ricerca scientifica
25 €	Donazione NN per la ricerca scientifica
4.000 €	Donazione NUOVO PIGNONE SRL per la ricerca scientifica

SUPPORT US

Any amount you want donate for the research will be a real help in the fight against the beta-sarcoglycanopathies and the other forms of muscular dystrophy.

FUND FOR RESEARCH:

For this purpose a special **FUND FOR RESEARCH** was created on its bank account of Banca Prossima. All donations on the account will be used to finance theAmerican projects and the scientific researches on the LGMD2E-2D-2C-2F.

HEADING FOR RESEARCH FUND: Gruppo Familiari Beta-sarcoglicanopatie Onlus

IBAN: IT33X0335901600100000076500

BIC /SWIFT   code   BCITITMX

PAYPAL TRANSFER

HEADING: Gruppo Familiari Beta-sarcoglicanopatie Onlus

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