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Updates GFB n. 1 - January 2021

SAREPTA THERAPEUTICS INVESTIGATIONAL GENE THERAPY SRP-9003 FOR THE TREATMENT OF LIMB-GIRDLE MUSCULAR DYSTROPHY TYPE 2E SHOWS SUSTAINED FUNCTIONAL IMPROVEMENTS 18-MONTHS AFTER ADMINISTRATION

9/28/20 -- Continued functional improvements were observed at 18 months in the low-dose cohort -- -- First look at functional outcomes in high-dose cohort found improvements 6 months after administration -- -- Results in both cohorts continue to reinforce safety and tolerability profile of SRP-9003

-- CAMBRIDGE, Mass., Sept. 28, 2020 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced positive results from the ongoing study of SRP-9003 (rAAVrh74.MHCK7.hSGCB), the Company's investigational gene therapy for limb-girdle muscular dystrophy Type 2E (LGMD2E). Results included 18-month functional data from three clinical trial participants in the low-dose cohort and 6-month functional data from three participants in the high-dose cohort.

SRP-9003

SRP-9003 is in development for the treatment of LGMD2E (also known as beta-sarcoglycanopathy and LGMDR4),

It's very encouraging that we continue to see consistent, positive data from our investigational gene therapy SRP-9003 across several measures, as we know the community needs more options," said Louise Rodino-Klapac, Ph.D., senior vice president of gene therapy, Sarepta Therapeutics. "The improvements in functional measures at 18- and 6- months in participants from both cohorts who received SRP-9003 are distinctly different from what an age-matched, natural history group would predict with LGMD2E. This sustained durability of the response in functional outcomes reinforces that SRP-9003 is getting to the muscle and suggestive of improvement against disease-mediated muscle damage.

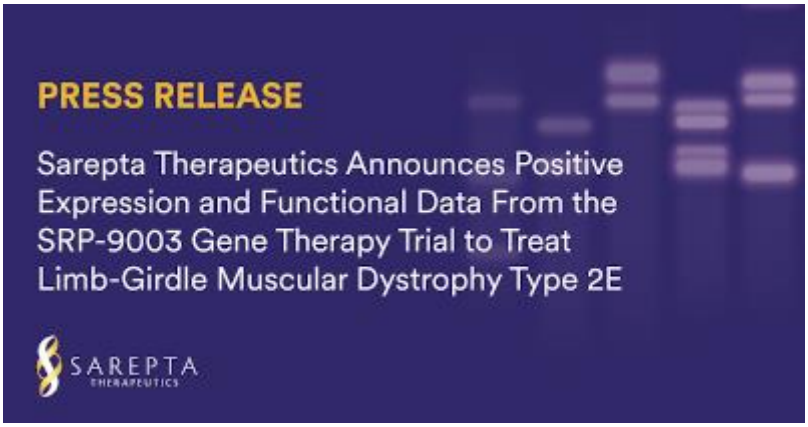
When coupled with the strong expression results and encouraging safety profile seen to date, today's results increase our confidence in the construct and provide additional evidence as we advance the higher dose of SRP-9003 into the next stage of clinical testing." Efficient transduction in skeletal muscle and robust beta-sarcoglycan protein expression were seen in both dose cohorts following infusion with SRP-9003, and significant creatine kinase (CK) reductions were observed at 90 days.

Cohort-specific results as follows:

Cohort 1 (low dose), at 18 months: All three participants continued to show improvements from baseline across all functional measures, including the North Star Assessment for Dysferlinopathies (NSAD), time-to-rise, four-stair climb, 100-meter walk test and 10-meter walk test. The mean NSAD improvement from baseline was 3.0 at 6 months and 5.7 at 18 months. There have been no new drug-related safety signals observed since the one-year update in June 2020, and no decreases in platelet counts outside of the normal range or signs of complement activation were observed.

Cohort 2 (high dose), at 6 months: All three participants demonstrated improvements from baseline across all functional measures, including the NSAD, time-to-rise, four-stair climb, 100-meter walk test and 10-meter walk test. The mean NSAD improvement from baseline was 3.7. There have been no new drug-related safety signals observed since expression results were shared in June 2020, and no decreases in platelet counts outside of the normal range or signs of complement activation were observed.

Press Release: https://investorrelations.sarepta.com/node/19961/pdf



LIMB GIRDLE MUSCULAR DISTROPHIES CAB ACTIVITIES HAVE STARTED

GFB took part to the creation of the Limb Girdle Muscular Diseases CAB (Community Advisory Board). The CAB is a sort of consortium that takes care of the new drug development, by creating interactions between specialized physicians and industries.

7 online CAB meetings have been held since 10th July 2020 and the Eurordis Summer School training course for CAB members has started and it will last 8 months.

In February, CAB meetings with companies dealing with Limb Girdle Muscular Diseases drug development, with the idea that a drug that works for a type of Limb Girdle Muscular Disease, could be used for a similar LGMD.

https://www.eurordis.org/content/eurordis-community-advisory-board-cab-programme

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THE NEW GFB INTERNET WEBSITE IS ONLINE

This is the new GFB internet website in Italian, that you can see at the link [www.gfbonlus.it.](http://www.gfbonlus.it)

The new international GFB internet website, in English, has been created at the link www.lgmd2e.org.

SAREPTA COMMUNITY BULLETIN: COVID-19 VACCINATION AND GENE THERAPY

Sarepta has received many questions related to COVID-19 vaccines and gene therapy. Here, Sarepta provide current answers to some of these frequently asked community questions. Answers are based on the information available as far as December 18th, 2020, and are subject to change based on new information. For more on COVID-19 vaccines, please view the CDC's COVID-19 website.

https://www.sarepta.com/sites/sarepta-corporate/files/2020-12/Community%20Bulletin_COVID19.pdf

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PARENT PROJECT USA: WATCH COVID-19 VACCINATION & DUCHENNE WHAT YOU NEED TO KNOW

We all have spent the last several months doing our best to keep ourselves and our families safe, while adjusting to this new virtual world. PPMD recognizes this has been nothing short of challenging, and we remain committed to providing support, community engagement, and the most accurate and up-to-date information possible during a time of unknowns. This extends to new learnings of COVID-19 vaccines, which PPMD believes to be the best form of protection against the virus, and we hope is a potential solution to alleviate this pandemic.

https://www.parentprojectmd.org/watch-covid-19-vaccination-duchenne-what-you-need-to-know-webinar-recording/

FROM PARENT PROJECT ITALIA : VACCINES AGAINST COVID-19 AND DUCHENNE

VACCINES AGANST COVID-19 AND DUCHENNE: WHAR YOU NEED TO KNOW

In the last few months, we've heard a lot about the several projects for the preparation of vaccines against the SARS-CoV-2 virus and about their efficacy results from their phase 3 studies. Two pharmaceutical companies, Pfizer-BioNTech e Moderna Therapeutics have asked for authorization to the emergency use (EUA) to FDA hoping to be authorized within the end of 2020. On December 10th, FDA Advisor Board for vaccines and related organic products (VRBPAC) of the USA have voted in support of granting the authorization for the Pfizer-BioNTech BNT162b2 vaccine.

Moderna Therapeutics has a scheduled meeting on Thursday 17th December.

Read the complete information at the link:

http://parentproject.it/2020/12/15/i-vaccini-contro-il-covid-19-e-la-duchenne-cosa-ce-da-sapere/

TWO ONGOING NATURAL HISTORY STUDIES FOR SARCOGLYCANOPATHIES

In 2020 due natural history studies on Limb Girdle Muscular Disease type 2C-2D-2E.

The first study has been Italian, carried out at the Policlinico in Milan by Professor Yvan Torrente and it involves 33 patients affected by LGMD2E.

The second study is in progress in the United States for patients affected by LGMD2C-2D-2E

All the information on the ongoing studies can be found on the GFB website at the following link:

http://www.beta-sarcoglicanopatie.it/ricerca-scientifica/archivio-pubblicazioni-scientifiche/studi-clinici.html

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AN ARTICLE CONCERNING 439 PATIENTS AFFECTED BY LGMD 2C-2D-2E HAS BEEN PUBLISHED.

In September the article “New genotype-phenotype correlations in a large European cohort of patients with sarcoglycanopathy” concerning 439 patients coming from 13 European countries, affected by Sarcoglycanopathy was published.

The project coordinator is professor Jordi Diaz Manera.

For a deepening, go to the link : https://pubmed.ncbi.nlm.nih.gov/32875335/

THE FIRST GENETHON BIOTECH FOR LGMD

The Généthon team, the no profit laboratory of the French AFM-Telethon, have been working for many years on an innovative project.

This biotech that has being created, called **Atamyo Therapeutics**, will have, at the beginning, the aim of developing products for the most common limb girdle muscular diseases and sell the at a fair and controlled price.

In 2021 a first clinical trial for LGMD linked to FKRP (LGMD R9 / ex. LGMD 2I) is supposed to start.

The following year it would be calpainopathy (LGMD R1 / ex. LGMD 2A) and gamma-sarcoglicanopathy (LGMD R5 / ex. LGMD 2C) turn.

Then in 2023. It would be the disferlinopathy (LGMD R2 / es. LGMD 2B) and alfa-sarcoglicanopathy (LGMD R3 / es. LGMD 2D) turn.

Read the complete information at the link: https://lgmd.afm-telethon.fr/premiere-biotech-de-genethon/

WEBINAR ON MUSCULAR DYSTROPHIES , THE VOLUNTEERING AND THE SCIENTIFIC RESEARCH

2020 has been the year for cooperation and online events. All the year long, a lot of webinars, in Italian and in English have been provided and they have been reported on GFB website to the following link:

http://www.beta-sarcoglicanopatie.it/9-news/191-webinar-sulle-distrofie-dei-cingoli-e-la-ricerca-scientifica.html

http://www.beta-sarcoglicanopathy.org/9-news/202-international-webinar.html

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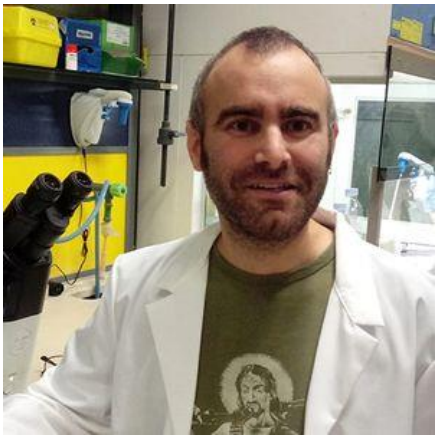
GFB HAS ORGANIZED THE INTERNATIONAL EVENT "LET'S MEET"

LET'S MEET has been the first international meeting of the Limb Girdle Muscular Diseases patient organizations.

The event has been organized by GFB, involving **17 patient foundations coming from 14 states** in Europe**,** , Asia, Africa e America. GFB is working with the other patient organizations for the creation of a global net for the Limb Girdle Muscular Dystrophies. In 2021 a second LET'S MEET online meeting will be held.

INTERVIEW TO CARLES SANCHEZ RIERA AT RADIO FINESTRAPERTA

Carles Sanchez Riera, researcher and GFB member, has been interviewed by radio FinestrAperta.



You can listen to the interview at the link:
https://www.mixcloud.com/FinestrAperta/08072020-il-granello-di-sale-lintervista-a-carles-s%C3%A1nchez-riera/

STEFANIA'S WITNESS

The UILDM family is composed by different types of volunteers. Stefania Pedroni, national vice president, not only represents an institutional figure but she is also a psychologist at the NEMO Center in Milan and she's also a woman who shares with her associations her human and professional skills. Hereafter, she speaks about the importance of the psychological support for people affected by dystrophy and their families. During the lockdown, she set up a Psychological Support Service that still continues.

Read the complete information at the link: https://www.uildm.org/il-volontariato-%C3%A8-crescita

THE ITALIAN LIMB GIRDLES GROUP IS BORN

The UILDM Girdles Group, coordinated by the national vice president Stefania Pedroni, by its members Alba De Santis from Turin, Carles Sanchez Riera and Manuel Tartaglia from Rome and Riccardo Rutigliano from Milan.

The aim of the Group is to create a discussion inside UILDM to follow the new therapeutic developments, to network with the other European organizations and to updates all the members.

Read all at the following link: https://www.uildm.org/%C3%A8-nato-il-gruppo-cingoli-uildm

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CONTRIBUTIONS AND DONATIONS RECEIVED BY GFB IN THE LAST MONTHS

12,70 €	Donation NN for the scientific research from Chieti
14.687,74 €	5x1000 Contribution concerning 2019
120 €	Donation NN for the scientific research from Roma
2.000 €	Donation NN for the scientific research from Lecco
10.000 €	Sarepta contribution for Telemedicine project
275 €	Donation NN from Belgium
200 €	Donation NN for the scientific research from Spain
150 €	Donation NN for the scientific research

GFB PATIENTS AFFECTED BY LGMD 2C-2D-2E

In total, so far, GFB is composed by 545 patients affected by Sarcoglicanopatia, divided as follows:

	LGMD2C	LGMD2D	LGMD2E	LGMD2F	SARCOGL.
2010	0	1	5	0	
2013	4	14	14	1	
2014	9	27	21	1	
2015	12	50	25	1	
2016	23	77	69	1	3
2017	37	104	97	4	3
2018	132	152	112	4	3
2019	145	184	134	5	3
2020	164	227	146	5	3
