THE GENE THERAPY PROJECT FUNDED BY GFB ONLUS

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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 is in development for the treatment of LGMD2E (also known as beta-sarcoglycanopathy and LGMDR4), a devastating monogenic neuromuscular disease caused by a lack of beta-sarcoglycan proteins. SRP-9003 is a gene construct that transduces skeletal and cardiac muscle, delivering a gene that codes for the full-length beta-sarcoglycan protein, the absence of which is the sole cause of the progressive degeneration and a shortened lifespan characterized by the disease. "There are currently no approved treatments for people with LGMD2E – a disease that causes significant disability in children and often leads to early mortality.

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.

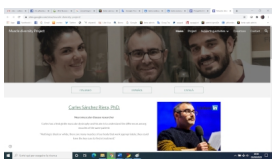
read more

"MUSCLE DIVERSITY": THE CARLES SANCHEZ RIERA PROJECT

Carles Sanchez Riera is starting an interesting project on LGMD Limb Girdle Muscular Dystrophies, entitled "Muscular Diversity", at the "La Sapienza University" of Rome, in Italy.

Carles has a limb girdle muscular dystrophy and his aim is to understand the differences among muscles of the same patient: "Nothing is black or white, there are many muscles of our body that work appropriately, they could have the key cues to find a treatment."

https://sites.google.com/view/muscle-diversity-project/



The GFB on National Television for the seventh time

On July 24, Rai Parlamento broadcast the seventh interview of the GFB on the Italian national television, entitled "Beta-sarcoglicanopatie: research and telemedicine".

watch the video

POSITIVE DATA FOR SRP-9003

June 8th 2020: Sarepta Therapeutics Announces Positive Expression and Functional Data From the SRP-9003 Gene Therapy Trial to Treat Limb-Girdle Muscular Dystrophy Type 2E.

Read more

Other press releases:

New data gives hope for Limb Girdle Muscular Dystrophy gene therapy - Patient Worthy

SRP-9003 gene therapy for LGMD2E shows benefits after 1 year - Muscular dystrophy news today

COVID-19 RESOURCES FOR MUSCULAR DYSTROPHY COMMUNITY

FROM THE SPEAK FOUNDATION:

In the website of the Speak Foundation www.thespeakfoundation.com you can find:

- COVID-19 Advocacy Letter for Individuals with MD

- Advocacy Letter to Healthcare Providers

- COVID-19 Preparedness Checklist It is extremely important that you gather the following information and have it readily available if you should become ill from COVID-19. You should also make sure your family members and all caregivers know where this information is kept in the event you require going to the hospital for health issues related to COVID-19. Keep it in a safe and easily accessible location.

FROM EURORDIS

In the website of Eurordis https://www.eurordis.org/content/eurordis-press-releases you can find:

- E URORDISstatement_COVID19Triage

EURORDIS-Rare Diseases Europe would like to bring to their urgent attention the concerns and needs of people living with a rare disease, their families and carers during the COVID-19 crisis. Through this open letter, we implore policy makers and authorities in Europe and around the world to take action to protect people living with a rare disease from becoming even more vulnerable during this crisis in line with the recommendations set out.

CORONAVIRUS: informations for MD patients

Here you can find PPMD COVID-19 (CORONAVIRUS) INFORMATION CENTER

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 403 patients affected from Sarcoglycanopathy, so divided:

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
2020	158	206	141	5

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

SUPPORTERS

2020

1.000 €

Donazione NN per la ricerca scientifica

15.104,23	€	Contributo 5x1000 relativo al 2018
50 €		

Donazione NN per la ricerca scientifica da Salerno

120 €

Donazione NN per la ricerca scientifica

15 €	Donazione NN per la ricerca scientifica
20 €	Donazione NN per la ricerca scientifica
10 €	Donazione NN per la ricerca scientifica dalla Spagna
2000 €	Donazione NN per la ricerca scientifica da Lecco
150 €	Donazione NN per la ricerca scientifica
30 €	Donazione NN per la ricerca scientifica
200 €	Donazione NN per la ricerca scientifica
100 €	Donazione NN per la ricerca scientifica

2019

300 €	Donazione NN per la ricerca scientifica	
1.000 €	Donazione NN per la ricerca scientifica da Roma	
150 €	Donazione NN per la ricerca scientifica	
200 €	Donazione NN per la ricerca scientifica	
13.424,23	€	Contributo 5x1000 relativo al 2017
150 €	Donazione NN per la ricerca scientifica	
20 €	Donazione NN per la ricerca scientifica	
50 €	Donazione NN per la ricerca scientifica da Treviso	

85,21 €	Donazione Pancaffè per la ricerca scientifica
250 €	Donazione coscritti 1949 di Talamona in memoria di Vola Bruno
2.945 €	Donazione Memorial Simone Alberton da Treviso
300 €	Donazione NN per la ricerca scientifica da Roma
200 €	Donazione NN per la ricerca scientifica
1.130 €	Donazione NN per la ricerca scientifica da Como
135 €	Donazione coscritti 1939 di Talamona in memoria di Tirinzoni Guido
600 €	Donazione NN per la ricerca scientifica
300 €	Donazione NN per la ricerca scientifica da Viareggio
50 €	Donazione NN per la ricerca scientifica da Roma
300 €	Donazione NN per la ricerca scientifica da Roma
1.000 €	Donazione NN per la ricerca scientifica
2.000 €	Donazione NN per la ricerca scientifica da Lecco
110 €	Donazione coscritti 1964 di Talamona
150 €	Donazione NN per la ricerca scientifica
300 €	Donazione NN per la ricerca scientifica da Lecco
250 €	Donazione NN per la ricerca scientifica
150 €	Donazione NN per la ricerca scientifica da Roma
25.549,44	**TOTALE 2019**

OTHER SUPPORTERS 2018-2017-2016-2015-2014-2013

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