GFB

Beta-Sarcoglycanopathy Family Group

Via Civasca 112, 23018 Talamona (SO) Italy

Cell. 3280075986, email info@beta-sarcoglicanopatie.it

www.beta-sarcoglicanopatie.it

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MYONEXUS THERAPEUTICS SECURES $2.5 MILLION SEED FINANCING TO CLINICALLY ADVANCE LIMB-GIRDLE MUSCULAR DYSTROPHY (LGMD) GENE THERAPIES

MYO-101 Phase 1/2a systemic gene therapy trial for LGMD2E to begin in early 2018.

NEW ALBANY, Ohio -- Myonexus Therapeutics, Inc. (Myonexus), a clinical-stage biotechnology company developing transformative gene therapies for limb-girdle muscular dystrophies (LGMDs), **today announced the completion of a $2.5 million seed financing.** The company is committed to accelerating development of five gene therapies pioneered within the lab of Louise Rodino-Klapac, Ph.D. and under clinical guidance by Jerry Mendell, M.D., each a Principal Investigator at Nationwide Children's Hospital Center for Gene Therapy in Columbus, Ohio, USA. CincyTech, LLC, Rev1 Ventures, The Jain Foundation, and GFB ONLUS joined initial investors from the LGMD community.

The seed financing **will enable Myonexus to initiate a systemic Phase 1/2a clinical trial of MYO-101**, the company's gene therapy candidate for treating LGMD2E (beta-sarcoglycanopathy), in early 2018. Myonexus' pipeline also includes MYO-102, a gene therapy candidate for LGMD2D (alpha-sarcoglycanopathy) currently completing a Phase 1/2a clinical trial, MYO-201, a gene therapy candidate for LGMD2B (dysferlinopathy) currently in Phase 1, MYO-103, a preclinical gene therapy candidate for LGMD2C (gamma-sarcoglycanopathy), and MYO-301, a preclinical gene therapy candidate for LGMD2L (anoctamin 5).

"MYO-101's compelling preclinical data strongly supported the case for clinical translation, validated by our own subsequent clinical trial results as well as general advances in the neuromuscular disease gene therapy field," said Michael Triplett, PhD, Myonexus' President and Chief Executive Officer. "We are committed to rapidly advancing our pioneering pipeline of gene therapy candidates, with the ultimate goal of providing first-ever corrective LGMD treatments, and we plan to initiate the Phase 1/2a study of MYO-101 next year."

"We have a history of investing in world class pediatric innovations in partnership with Cincinnati Children's Hospital," said John Rice, PhD, Director of Life Sciences at CincyTech. "We are pleased to find another investment with an immediate and lasting impact on children living with these challenging and rare diseases. Nationwide Children's deserves great credit for developing this technology in house and proving it out clinically."

"There are currently no approved treatments for the limb-girdle muscular dystrophies. **Initial LGMD human clinical studies demonstrated expression in muscles exposed to MYO-102 and MYO-201**. MYO-101's Phase 1/2a clinical trial represents the first intravenous systemic exposure, potentially providing the first evidence of functional improvements in LGMD patients following treatment with these gene therapies," said Bruce Halpryn, PhD, Myonexus' Chief Operating Officer.

"With a ground-breaking approach Myonexus is poised to transform the way limb-girdle muscular dystrophy is treated, and we are thrilled to help accelerate their growth," said Tom Walker, CEO of Rev1 Ventures. "This news underscores the critical role that the Research Institute at Nationwide Children's Hospital is playing in advancing drug development research and identifying partners with the determination and ability to

commercialize these discoveries for the ultimate benefit of patients. We believe the partnership with Myonexus is setting the stage for big things in gene therapy."

"The Jain Foundation is excited to continue its support of the dysferlin gene therapy technologies emerging from Nationwide Children's and be part of the clinical development efforts happening at Myonexus. We believe gene therapy has great potential to be the first effective therapy for LGMD2B patients and to dramatically improve their quality of life," said Doug Albrecht, PhD, Co-President of the Jain Foundation Inc.

About Myonexus Therapeutics

Myonexus Therapeutics is a clinical stage, rare disease gene therapy company developing first ever treatments for Limb-girdle muscular dystrophies (LGMDs) based on research at Nationwide Children's Hospital, a leader in neuromuscular gene therapy discovery and translational research. Myonexus Therapeutics' pipeline includes three clinical stage gene therapy programs (LGMD2E, LGMD2D, and LGMD2B) and two preclinical gene therapy programs (LGMD2C and LGMD2LO). Founded in 2017, Myonexus is headquartered in New Albany, Ohio. More information is available at www.myonexustx.com.

About CincyTech LLC

CincyTech, one of the Midwest's most active seed investors, helps to transform innovation into high performing life science and digital companies in Southwest Ohio. Our team provides advice and seed capital to entrepreneurs, helps research institutions commercialize technology through startups, and catalyzes investment from individuals and institutions into regional companies. Learn more at http://cincytechusa.com.

About Rev1 Ventures

Rev1 is a venture fund that helps entrepreneurs build great companies. Combining investment capital with a unique blend of services through our startup studio, we propel innovation for startups and corporate innovation teams. Our seasoned, data-driven team helps lay the foundation for scalable growth with the skills to evolve a product, sell to customers, and build the right team. Named a top VC investor in the Great Lakes Region, Rev1 manages a continuum of financial support from corporate and community partners, as well as the Ohio Third Frontier. Rev1 was named the Most Active VC in Ohio in 2017 by CB Insights. For more information, visit http://www.rev1ventures.com.

About the Jain Foundation

The Jain Foundation is a privately funded nonprofit foundation focused on finding a cure for muscular dystrophies caused by dysferlin deficiency (LGMD2B/Miyoshi Myopathy). The Jain Foundation supports academic research, conducts independent studies with contract research organizations, and supports clinical studies in pursuit of this mission. The foundation also assists patients in receiving a confirmed genetic diagnosis of dysferlinopathy, and maintains a patient registry for the disease.

PUBLISHED IMPORTANT RESULTS FOR SMA1 TRIAL

On November 2nd the magazine The New England Journal of Medicine published the article "Single-Dose Gene-Replacement Therapy for Spinal Muscular Atrophy", the authors are K. Kaspar e J. Mendell. In the article there are all the results of the SMA1 trial of gene therapy.

See the article at the link: http://www.nejm.org/doi/full/10.1056/NEJMoa1706198#.WfsctKM_52d.facebook

Spinal muscular atrophy type 1 (SMA1) is a progressive, monogenic motor neuron disease with an onset during infancy that results in failure to achieve motor milestones and in death or the need for mechanical ventilation by

2 years of age. They studied functional replacement of the mutated gene encoding survival motor neuron 1 (SMN1) in this disease.

Fifteen patients with SMA1 received a single dose of intravenous adeno-associated virus serotype 9 carrying SMN complementary DNA encoding the missing SMN protein. Three of the patients received a low dose, and 12 received a high dose. The primary outcome was safety. The secondary outcome was the time until death or the need for permanent ventilatory assistance.

As of the data cutoff on August 7, 2017, all 15 patients were alive and event-free at 20 months of age, as compared with a rate of survival of 8% in a historical cohort. Of the 12 patients who had received the high dose, 11 sat unassisted, 9 rolled over, 11 fed orally and could speak, and 2 walked independently.

In patients with SMA1, a single intravenous infusion of adeno-associated viral vector containing DNA coding for SMN resulted in longer survival, superior achievement of motor milestones, and better motor function than in historical cohorts. Further studies are necessary to confirm the safety and efficacy of this gene therapy.

FDA CLEARS IND APPLICATION FOR MICRO-DYSTROPHIN GENE THERAPY PROGRAM TO TREAT DUCHENNE

The U.S. Food and Drug Administration (FDA) has cleared an investigational new drug application for a micro-dystrophin gene therapy program by Sarepta Therapeutics and Nationwide Children's Hospital, in clinical development for Duchenne muscular dystrophy (DMD).

A Phase 1/2a clinical trial is now enrolling participants and should begin dosing later in december.

The study will assess the safety and tolerability of AAVrh74.MHCK7.micro-Dystrophin in DMD patients. The trial is also designed to evaluate biological activity and efficacy of the micro-dystrophin vector by seeing how it performs when replacing the missing muscle dystrophin in DMD.

Between 60 to 70 percent of Duchenne boys could be potential candidates for this treatment, since researchers are looking for patients with mutations between exons 18 and 58.

For the trial, 12 patients will be split into two groups to undergo gene transfer and establish maximum tolerated dose (MTD) avoiding toxicity. One group will include infants three months to three years of age with DMD; the second will boys 4 to 7 years old.

The principal investigators, Dr. Jerry Mendell and Louise Rodino-Klapac of Nationwide Children's in Columbus, Ohio, will conduct the Phase 1/2a study. It'll use a construct (MHCK7) developed by Mendell and Rodino-Klapac to enhance activity of the micro-dystrophin vector in DMD patients. The construct can be delivered to skeletal, diaphragm and cardiac muscles and has shown high levels of gene expression in preclinical studies. Micro-dystrophin expression will be measured at three months, via biopsy.

This is the first micro-dystrophin gene therapy program in development for DMD. If approved, Sarepta has the option to retain worldwide exclusive rights.

"This will be the first clinical trial for DMD to treat patients as young as three months old, and is an important step forward in our quest to diagnose patients through newborn screenings," Mendell, who also heads the neuromuscular gene therapy program at Nationwide, said in a press release. "We are very encouraged by the promising preclinical results and are eager to complete enrollment."

Added Rodino-Klapac, principal investigator at Nationwide's Center for Gene Therapy: "With this clinical trial, we are laser-focused on giving each patient the best possible chance of a successful outcome. We are taking a

novel approach to trial design. The use of potentially therapeutic doses is a critical part of our mission to arm our patients for their one shot at gene therapy."

The clinical trial received $2.2 million in funding from Parent Project Muscular Dystrophy (PPMD). Recently, Sarepta and Nationwide announced the clearance of another IND application, regarding its GALGT2 gene therapy program for DMD.

"As the first systemic micro-dystrophin gene therapy program enters the clinic," said Sarepta President and CEO Douglas Ingram, "we mark an important milestone in our journey to relentlessly pursue new therapies to treat DMD."

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 222 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	29	96	93	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 RECEIVED OTHER CONTRIBUTIONS

The GFB Onlus has received the following contributions:

2017:

600 €	Donazione NN per la ricerca scientifica da Treviso
3.000 €	BIM Bacino Imbrifero Montano dell'Adda Sondrio
5.000 €	Contributo Ente privato
170 €	Donaz. coscritti 1940 Talamona in memoria di Ciaponi Enza
20 €	Donazione NN per la ricerca scientifica
30 €	Donaz. coscritti 1945 Talamona in memoria di Sutti Armanda
2.539 €	Donazione NN per la ricerca scientifica da Dongo
160 €	Donaz. coscritti 1941 Talamona in memoria di Ruffoni Egidio

450 €	Donazione NN per la ricerca scientifica da Lecco
80 €	Donazione colleghe di Linda Camero
80 €	Donazione coscritti 1942 di Talamona 75° anniversario
75 €	Donazione NN per la ricerca scientifica dalla Francia
1.000 €	Contributo ente privato
200 €	Donazione NN per la ricerca scientifica dalla Francia

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

EMAIL: info@beta-sarcoglicanopatie.it