MDA CLINICAL & SCIENTIFIC CONFERENCE

Insights in Research Investor Summit

1st Annual MDA Insights in Research Investor Summit (IRIS) for Neuromuscular Disease

Thursday, April 29 – Friday, April 30, 2021

MDA Muscular Dystrophy Association

1st Annual MDA Insights in Research Investor Summit (IRIS) for Neuromuscular Disease

All times listed are Eastern Standard Time

MDA CLINICAL & SCIENTIFIC CONFERENCE 2021
Insights in Research Investor Summit

To view the presentations and recordings, visit the IRIS website here

Thursday, April 29, 2021	
10:15-10:30 AM	Opening Remarks
Licensing Opportunities	
10:30-10:45 AM	***One Molecule, Two Therapeutic Applications*** Luis Carlos Lopez Garcia, PhD University of Granada (UGR)
10:45-11:00 AM	***New Strategy to Treat Sarcoglycanopathies: CFTR Correctors for Recovering Misfolded Proteins*** Dorianna Sandona, PhD University of Padova
11:00-11:15 AM	***Prosetin, A New Investigational Drug for ALS, Protects Motor Neurons from Misfolded Proteins*** Hynek Wichterle Columbia University
11:15-11:30 AM	***Novel siRNA Muscle Targeting Platform: A Potential Treatment for FSHD*** Abbas Abdallah, PhD UMASS Medical School
11:30-11:45 AM	***GsMTx4 Treatment for Muscular Dystrophy*** Thomas M. Suchyna, Ph.D. SUNY at Buffalo
Disease-Focused Venture Philanthropy and Venture Capital Funds	
11:45 -11:55 AM	Cure Duchenne Venture Philanthropy Lianna Orlando, PhD
11:55 AM-12:05 PM	ALS Investment Fund Craig Boyce
12:05-12:15 PM	MDA Venture Philanthropy Sharon Hesterlee, PhD
12:15-12:45 PM	Break for Lunch
Trends in NMD Development and Commercialization – Cello Health	
12:45-1:45 PM	** Moderator: Michael C. Rice, VP, Head of Advanced Therapeutics, Cello Health BioConsulting ***Investment in Therapeutic Innovations for Genetic Disorders – A Brainstorm for the Overall Neurology and Musculoskeletal Sectors*** Invited Panelists: • Chris Garabedian - Xontogeny and Portfolio Manager, PXV Fund • Beth Seidenberg – Westlake Village BioPartners • Sharon Hesterlee, PhD – Muscular Dystrophy Association • Lea Hachigian – Longwood Fund • Inbal Michailovici – Futurx

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Thursday, April 29, 2021 Continued	
Investment Opportunities	
1:45-2:05 PM	***Anti-misfolding Small Molecules Targeting TDP Isoforms in ALS*** Chris Barden CEO Treventis
2:05-2:25 PM	***Matrix Metalloproteinase Inhibitor for the Treatment of Amyotrophic Lateral Sclerosis*** Irving Sucholeiki President Aquilus Pharmaceuticals
2:25-2:45 PM	***Novel Protein Folding Gene Therapy for ALS*** Akinori Hishiya Principal Scientist Sola Biosciences
2:45-3:05 PM	***Small Molecule Assembly Modulators: A Novel Approach to ALS Therapeutics*** Vishwanath R. Lingappa, MD, PhD CEO & CTO Prosetta Biosciences
3:05-3:25 PM	***Advancing Novel Investigational Drugs for the Treatment of Sporadic Amyotrophic Lateral Sclerosis*** J. P. Pearson, Ph.D. CEO, CSO Sea Pharmaceuticals
3:25-3:45 PM	***Treating Incurable Neurodegenerative Diseases with an Integrated Technology Platform and a Synergistic Approach*** Antonella Favit-VanPelt, MD, PhD President & Chairwoman of the Board TheraNeuropharma
3:45-4:05 PM	***A Combination Therapy Approach to Treating ALS*** Anjan (AJ) Aralihalli, Founder Raya Therapeutics
4:05-4:25 PM	***Advanced Predictive Analytics for Drug Rescue*** David Ennist, PhD, MBA, CEO & Chief Science Officer Origent Data Sciences
4:25-4:45 PM	***A Novel Non-Invasive Neuromodulation Approach to Treatment of ALS Using Neuronal Hyperexcitability Suppression*** Nader Yaghoubi, M.D., Ph.D. – President and CEO PathMaker Nuerosystems Inc
4:45-5:05 PM	***IXC-109, the first drug to rescue multiple animal models of Mitochondrial Orphan Disease*** Gino Cortopassi, PhD CEO Ixchel Pharma

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Friday, April 30, 2021	
10:00-10:15 AM	Opening Remarks
Licensing Opportunities	
10:15-10:30 AM	***Cellular Medicine for Skeletal Muscle Wasting and Disease*** Michael Hicks, PhD UC Irvine
10:30-10:45 AM	***D3creatine dilution: A Direct, Accurate, and Non-Invasive Measurement of Functional Muscle Mass*** William J Evans, PhD UC Berkeley
10:45-11:00 AM	***Extracellular vesicles for monitoring mRNA biomarkers of muscular dystrophies*** Thurman Wheeler, MD Massachusetts General Hospital
11:00-11:15 AM	***AI-enabled Insoles to Assess Gait Function in Persons with Neuromuscular Disease in Controlled and Real-life Environments*** Damiano Zanotto, PhD (Jacqueline Montes, PT, EdD) Stevens Institute of Technology
11:15-11:30 AM	***Screening Biomarkers – Tracking Inflammation and Oxidative Stress in Muscular Dystrophy*** Peter Arthur, PhD University of Western Australia
11:30AM-12:00 PM	Break for Lunch
MDA Investment Showcase	
12:00-12:15 PM	***Advancing Novel Gene Therapies: From Venture Philanthropy to Series A*** Barry Byrne, MD, PhD, Founder & Director AavantiBio
12:15-12:30 PM	***MyoGene Bio and our gene editing therapy for Duchenne*** Cortney Young, Co-Founder and CEO MyoGene
12:30-12:45 PM	***A Novel CD38 Approach to Directly and Simultaneously Protect Neurons, Protect Muscles and Handle Inflammation, by Activating Rescue Mechanisms of Suffering Cells*** Laurence Bressac, CEO Encefa
Investment Opportunities	
12:45-1:05 PM	***miRecule's DreamiR Platform: Development of Best-in-Class RNA Therapeutics for Muscular Dystrophy and other Disorders*** Anthony Saleh, CEO miRecule
1:05-1:25 PM	***A Non-Viral Platform for Targeted Delivery of Genes to Skeletal and Cardiac Muscle*** Stanley C. Froehner Co-founder and Chairman of the Board Myosana Therapeutics
1:25-1:45 PM	***Novel Utrophin-Upregulation Small Molecule Drugs for Duchenne Muscular Dystrophy (DMD)*** Tejvir S. Khurana, MD, PhD, Founder & CEO MyoArete
1:45-2:05 PM	***AVGN7, a Novel Gene Therapeutic for Inclusion Body Myositis*** Dan Rodgers, PhD, Founder & CEO AAVogen Inc.

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Friday, April 30, 2021 Continued	
2:05-2:25 PM	***Antibody-Based Delivery of Biologics for Heart- and Muscle-Targeted Treatment of Myopathies*** Robert Shaffer, Founder & CEO Enable Therapeutics
2:25-2:45 PM	***Mas/MrgD receptor agonists for the treatment of DMD*** Elizabeth Wagner, COO Constant Therapeutics
2:45-3:05 PM	***Delivery of Full-Length Dystrophin*** Joshua Selsby, PhD Extrave Biosciences
3:05-3:25 PM	***Regenerative Protein Therapeutics Derived from the Human Embryonic Stem Cell Secretome for Neuromuscular and Muscle Wasting Diseases*** Hanadie Yousef, PhD, CEO & Co-Founder Juvena Therapeutics
3:25-3:45 PM	***TOL2: A Drug Candidate for Antigen-Specific Immunotherapy of Myasthenia Gravis*** Charlotte Fribert CEO Toleranzia
3:45 PM	Closing Remarks

** **Investment in Therapeutic Innovations for Genetic Disorders – A Brainstorm for the Overall Neurology and Musculoskeletal Sectors**

Recent advances in genetic based modalities and immune modulating therapies for neuromuscular diseases (NMD) has brought the sector back to the forefront of Pharma R&D priorities. The realization of breakthrough disease modifying therapeutic platforms, such as protein replacement, nucleic acid therapeutics, small molecule mRNA splice modifiers and Gene Therapy, largely Adeno-associated Virus (AAV) based gene augmentation, has increase our understanding of neurological disease pathobiology and ways to modulate intractable targets and deliver to affected neuronal and muscle tissues. Such convergence of biology, technology, and clinical/regulator navigation, along with the immense influx of speculative capital makes 2021 the "perfect brainstorm" for innovation broader neurologic and muscular disorders. As such, therapeutic platforms for NMDs have recently become one of the most active area of acquisitions (e.g., Novartis/AveXis for $8.7B, Astellas/Audentes for $3B, Lilly/Prevail for $1B, Bayer/AskBio $2B) and strategic alliances (e.g., Biogen/Ionis $1B, Sarepta/Roche, $1.7B, PTC/Roche $490M, Takeda/StrideBio $680M). Such breakthroughs and Exits draw excitement for more early-stage, but potentially disease-modifying therapeutic approaches, to address a range of challenging disorders. Innovators and rising to the challenge with projects raising a record level of venture financing (Dynacure $110M, Neurogene $68.5M) and IPOs (Passage Bio $248M, Dyne, 233M, Taysha Gene Tx $181M, Avidity Bio $298M, Scholar Rock $75M).

Today's panel will discuss trends in innovation and the investment options for early-stage therapeutic developers to address the unmet needs of patients with NMDs and will provide insight into how investors evaluate disruptive therapies and whitespace therapeutic opportunities to make investment decisions.

ATTENDING INVESTORS

Attendee Name	Organization	Job Title
Albrecht, Douglas	Jain Foundation, Inc.	Co-President
Aralihalli, Anjan	CTI Life Sciences	Venture Partner
Armentano, Donna	Pfizer	Executive Director
Baker, Chirs	Advent Life Sciences	Associate
Balogh, Peter	RA Capital Management	Junior Associate
Barsher, Brian	Barsher & Associates	Principal
BOU DARGHAM, Daria	Genethon	Chargée Business Development
Boyce, Craig	ALS Investment Fund	Managing Director
Brady, Todd	Brace Pharma Capital	Director of Finance and Investments
Brennan, Christine	MRL Ventures Fund	Partner
Bryant, Kathryn	The Speak Foundation	Founder and CEO
Cabrera MD PhD, Gustavo	Global BioTherapeutics Inc	CEO
Camino, Eric	PPMD	VP Research and Clinical Innovation
Chappel, Amy	Eliem TX	CMP
Coco, Stefano	3B Future Health Fund (formerly Helsinn Investment Fund)	Investment Analyst
Columbano, Angela	Genethon	Business Development Head
Drew, Charlotte	Kurt+Peter Foundation	Treasurer
Dziewczapolski, Gustavo	Cure CMD	Scientific Director
Estess, Meredith	Project ALS	Co-Founder
Estess, Valerie	Project ALS	Director of Research
Fedorkova, Lenka	Harrington Discovery Institute	VP, Business Dev. & Strategic Alliances
Fleming, Erin	Project ALS	Director of Research
Frewing, Scott	Kurt+Peter Foundation	President
Garabedian, Chris	Xontogeny	Chairman & CEO
Gillespie, Michael	RA Capital Management	Associate
Glascock, Jackie	Cure SMA	Director of Research Programs
Gray, Amy	CMTA	CEO
Grint, Paul	The Highgate Group	CEO
Han, Steve	Takeda Pharmaceuticals	Senior Medical Director
Hansen, Philip	Heslin Rothenberg Farley & Mesiti	Partner
Hashimoto, Kentaro	Takeda Pharmaceuticals	Director

Healy, Aileen	Pfizer	Global Head, Emerging Science and Innovation-Rare Diseases
Heidecker, Martin	Boehringer Ingelheim Venture Fund USA	Managing Director
Hulbert, Meredith	Project ALS	Co-Founder
Jarecki, Jill	Cure SMA	CSO
Kavanaugh, Michael	McLaughlin Research Institute	CSO
King, Aidan	Fountain Healthcare Partners	Managing Partner
Kinsey, Bert	Pappas Capital	
Kishi, Yasuhiro	Mitsubishi Tanabe Pharma Holdings America	Director, External Innovation
Krench, Meg	Vertex Pharmaceuticals	Director, External Innovation
Krattenmaker, Matthew	Takeda	
Krishnan, Naveen	Bayer	Senior Director Venture Investments
Larkindale, Jane	PepGen	Sr. Director Clinical Sciences
LaRosa, Greg	Pfizer	VP
Levy, Jennifer	Coalition to Cure Calpain 3	Scientific Director
Leitner, Melanie	ALS Investment Fund	CSO
Lowery, William	LGMD-1D DNAJB6 Foundation	Director of Operations
McMath, Elizabeth	Novartis	Sr. Manager External Innovation
Melton, Roger	Inotiv	Director - DMPK
Okuno, Tsuyoshi	MP Healthcare Venture Management Inc.	Director
Owens, Steven	Cerebrasol Canada Ltd	Scientist
Qian, X.	Boston Pharmaceuticals	VP Global Medical Evaluations and Strategy
Piault, Salomé	Genethon	Business development & partnership
Ritzeler, Olaf	Sanofi	BD&L
Rufibach, Laura	Jain Foundation	Co-President
Ruth, Jason	5AM Ventures	Principal
Sagartz, John	Inotiv	Chief Strategy Officer
Thayer, Joshua	Jain Foundation	General Counsel
Tracy, Mark	Tracy BioConsulting, LLC	General Counsel
Tsura, Sayaka	Mitsubishi Tanabe Pharma Holdings America, Inc.	Associate Director
Williams, Brad	Jain Foundation	Director of Research
Wyckoff, Matthew	Aceras Life Sciences	Partner
Xavier, Asish	Johnson & Johnson Innovation - JJDC	VP, Venture Investments
Yasuda, Shinichiro	Takeda Pharmaceuticals	Sr. Director, Translational Medicine

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Cello Health BioConsulting: Who We Are

- Cello Health BioConsulting is a knowledge-based consultancy deeply rooted in science; we often evaluate early stage programs before much, if any, clinical data is available. In the biopharma world, we are known for our "unconventional insight" – forward thinking, independent, objective strategic advice across all therapeutic areas.
- Cello Health BioConsulting provides strategic advice for corporate growth and partnering strategies, disease area selection, indication prioritization, opportunity search and evaluation, opportunity and landscape assessment, valuation and forecasting, early market access strategy and early value profile development.
- Cello Health BioConsulting has a strong and broad network of leaders and influencers across biotech and pharma, which provides a deep understanding of next wave issues, the competitive and market landscapes, and keeps us well-informed and ahead of industry trends.
- Visit our website at www.cellohealthbioconsulting.com

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Previously Defined Health

CELLO HEALTH BioConsulting Previously Defined Health

MDA® Muscular Dystrophy Association

Investment in Therapeutic Innovations for Genetic Disorders – *A Brainstorm for the Overall Neurology and Musculoskeletal Sectors*

Moderator

Michael C. Rice, MS, MBA
VP, Head of Advanced Therapeutics, Cello Health BioConsulting

CELLO HEALTH BioConsulting

2021 MDA CLINICAL & SCIENTIFIC CONFERENCE
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Panelists

Chris Garabedian
Xontogeny and Portfolio Manager, PXV Fund

Beth Seidenberg
Westlake Village BioPartners

Sharon Hesterlee, PhD
Muscular Dystrophy Association

Lea Hachigian
Longwood Fund

Inbal Michailovici
FutureRx

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Disease-Focused Venture Philanthropy and Venture Capital Funds

CureDuchenne Venture Philanthropy

Debra Miller, Founder and CEO

CureDuchenne's portfolio includes wide-ranging projects aimed at finding treatments for Duchenne. This includes investments in companies pursuing dystrophin-restoring approaches as well as those developing anti-inflammatory and other mechanisms contributing to the disease. We also look for novel technologies and platform approaches aimed at overcoming the limitations of existing therapies in development. Investments from CureDuchenne Ventures have successfully leveraged follow-on financing from venture capital, biotech, and pharmaceutical companies, and have resulted in several successful exits.

ALS Investment Fund

Craig Boyce, Managing Director

ALS Investment Fund ("ALS-IF") is a for-profit, Venture Capital fund that invests in cutting edge biotech companies developing drugs with a particular focus on ALS. Born out of the vision of three Dutch patients in 2016, the ALS-IF invests with a mission to create a world without ALS.

The ALS-IF fills a funding gap facing early-stage ALS companies, bridging and translating philanthropic supported research to proof of concept in clinical trials. We look for companies with either platform approaches relevant to multiple diseases, and/or with multiple drugs already in clinical development. The companies in our portfolio have proven the basic science behind their approaches and are working towards validating their therapeutic value.

MDA Venture Philanthropy

Sharon Hesterlee, PhD, EVP & Chief Research Officer

MDA Venture Philanthropy (MVP) is the Muscular Dystrophy Association's drug development program, which operates within MDA's Translational Research program. MVP is exclusively focused on funding the discovery and clinical application of treatments and cures for neuromuscular diseases.

Adapting elements of the venture capital model, the MVP business plan is characterized by an emphasis on measurable results along with deep involvement by its scientific and industry advisers. MVP evaluates and makes targeted investments in for-profit and not-for-profit companies and academics developing therapeutics for neuromuscular diseases.

Building upon MDA's long-term investment in research and health care, MVP is designed to complement MDA's ongoing programs of health care, lifesaving services, advocacy, basic and clinical research, and professional and public health information. MVP also benefits from MDA's other research programs that support basic research, clinical trials and research infrastructure.

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ACADEMIC PROFILE

Institution

Columbia University

COLUMBIA UNIVERSITY IN THE CITY OF NEW YORK

Indication

Amyotrophic lateral sclerosis (ALS)

Stage

Preclinical

Description of Therapeutic

Prosetin is a highly potent, oral, brain penetrant, well-tolerated MAP4K inhibitor designed for the treatment of ALS. In screens of small molecule compound libraries using the *in vitro* screening platform described below, we found that compounds inhibiting the MAP kinase pathway are strongly protective against ER stress—the series of pathways that are activated when cells accumulate misfolded proteins, and one of the earliest molecular phenotypes that can be detected in ALS. Further computational analysis and genetic ablation of the shared targets of protective compounds in this assay identified MAP4Ks as the critical regulators of this pathway. We therefore developed analogs of one top performer, URMC-099, for enhanced MAP4K inhibition and optimized ADME properties including strong metabolic stability and exceptional brain penetrance, with prosetin emerging as the lead candidate.

In partnership with the non-profit Project ALS, we have completed *in vivo* efficacy and IND enabling studies for prosetin. We plan to submit an IND application for prosetin in Q2'21, and to initiate a hybrid Phase 1 clinical trial in both healthy volunteers and ALS patients soon thereafter should we receive regulatory approval.

Description of Platform

We developed an *in vitro* screening platform to address a fundamental challenge in ALS drug discovery: stem cell-derived motor neurons from people with ALS do not exhibit key hallmarks of the disease, and thus—despite clear cellular relevance to human ALS—their utility in drug screening has been limited. Further, while several studies have reported differences between wild-type and ALS motor neurons under various stress conditions, most of these stressors are non-specific, ill-defined, or both.

Thus, we set out to identify stressors that selectively potentiate ALS pathology. We screened for compounds that could exacerbate the survival differences between ALS and wild-type, and identified cyclopiazonic acid (CPA), a mycotoxin that induces the accumulation of misfolded proteins and activates ER stress, as an agent that is selectively lethal to ALS motor neurons. Because CPA treatment potentiates intrinsic, early imbalances in ALS motor neuron protein handling, it was an ideal candidate for building a scalable *in vitro* system to model a disease-relevant phenotype—and screen for compounds that rescue it.

IP Status

Confidential

Goals for Presentation

We are seeking partners for prosetin's clinical development. In a successful presentation, we will highlight differentiating features of this investment opportunity:

1. Prosetin was developed specifically for ALS. Despite research advances, ALS remains uniformly fatal and late-stage clinical trials continue to fail, partially due to a paucity of therapies rigorously developed with ALS as their lead indication. Prosetin's discovery and preclinical development was initiated and guided by a sophisticated investigation of ALS disease biology, and led by a multidisciplinary, dedicated team of ALS researchers. Our careful characterization of prosetin found that its primary MAP4K targets are directly linked to motor neuron survival, consistent with findings from ALS drug discovery groups at Genentech and Harvard.

2. Prosetin may be effective for all ALS patients, regardless of genetic background. Based on its mechanism of action, prosetin has the potential to benefit people with both heritable and sporadic forms of ALS, and we intend to evaluate its effects in a broad patient population.

3. Prosetin was optimized for ALS patients. Prosetin is orally bioavailable and highly brain-penetrant, allowing it to reach its targets in the CNS through oral administration. Prosetin also appears safe and well-tolerated throughout chronic treatment, which is essential for its intended use in ALS patients. To date, prosetin is the only published MAP4K inhibitor that meets these criteria.

Presenter Name

Hynek Wichterle

Website

http://stockwelllab.org, https://prosetin.org/

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ACADEMIC PROFILE

Institution

Massachusetts General Hospital

Indication

Measurement of molecular disease activity in muscular dystrophies

Stage

Clinical

Description of Therapeutic

N/A

Description of Platform

Extracellular RNA biomarkers of molecular disease activity that may be useful to detect drug target engagement in future clinical trials for muscular dystrophies.

PMID: 30254196 PMCID: PMC6156576 DOI: 10.1038/s41467-018-06206-0
PMID: 31211175 PMCID: PMC6562067 DOI: 10.1002/acn3.777

IP Status

Patent publication number WO/2018/017991

Goals for Presentation

Showcase licensing opportunity

Presenter Name

Thurman Wheeler

Website

https://www.massgeneral.org/neurology/research/wheeler-muscular-dystrophy-research-lab

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Timestamp: Day 2 1:01:10 – 1:15:12

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ACADEMIC PROFILE

Institution

Stevens Institute of Technology

STEVENS INSTITUTE of TECHNOLOGY
THE INNOVATION UNIVERSITY

COLUMBIA UNIVERSITY IRVING MEDICAL CENTER

Indication

Measurement of walking function

Stage

Clinical

Description of Therapeutic

As disease-modifying treatments are available or in development for many neuromuscular disorders (NMDs), showing encouraging results in motor and ambulatory function, there is a compelling need for sensitive measures of performance to assess gait function in these patients in real-life settings. Current body-worn sensors show promise for continuous gait monitoring in patients' living environments, but so far, they have produced modest accuracy. We devised novel instrumented insoles capable of measuring gait over multiple hours and in any environment. To achieve high accuracy, we developed machine learning (ML) inference models that substantially reduce measurement errors. Experiments with SMA and DMD patients support the feasibility of our approach. This research will pave the way for future clinical studies to characterize how gait function in NMDs is affected by disease-modifying treatments. In broader terms, the device provides an affordable measurement instrument for characterizing neuromuscular disorders affecting gait and balance.

Description of Platform

We have developed minimally obtrusive, AI-enabled smart insoles for accurate analysis of kinematic and kinetic gait parameters in controlled and free-living environments. The system consists of flexible, lightweight insoles embedding force and inertial sensors that can fit inside the patient's own shoes. A small plastic box is attached to the posterolateral side of each shoe with a small plastic clip. The box includes a battery and a processing unit with BLE connectivity and on-board data storage. In the current embodiment, the system can collect gait data at a selectable rate (333-500Hz), continuously, for up to 5 hrs. This high sample rate provides adequate temporal resolution to assess bilateral parameters (e.g., double support time) in healthy and pathological gait. The insoles can be synchronized with other instruments (e.g., EMG sensors) through a wireless board. The uniqueness of our technology lies in a new ML inference framework to improve validity and reliability. The framework generates personalized models without requiring subject-specific reference data to train the models, and therefore holds considerable potential for out-of-the lab and in-clinic gait assessments, for which laboratory equipment is often not available.

IP Status

We filed 2 patent applications (1 published, 1 provisional) on the technology. The new ML models enable high accuracy even when using mid-grade, cost-effective sensors. Our methods do not replace conventional data processing techniques. Instead, the outputs generated using conventional techniques are embedded into the ML models as domain knowledge and augmented with a subject-tailored subset of input features that substantially reduce measurement errors, allowing for more efficient learning from the same training dataset and sensor hardware.

Goals for Presentation

Objective 1 - Present interim results of our ongoing MDA project, supporting validity and reliability of the insole technology in measuring gait function in individuals with DMD and SMA. Summarize related studies from our group, providing further evidence of the applicability of this technology to clinical research.

Objective 2 - Present our go-to-market strategy, obtain useful feedback from the audience, and stir up interest from potential investors to support the development of an MVP, based on the current prototype, to study neuromuscular disorders affecting gait and balance.

Presenter Name

Damiano Zanotto (Stevens Inst., Engineering lead)
Jacqueline Montes (Columbia Univ., Clinical lead)

Website

Wearable Robotic Systems Lab. www.WRSlab.com

Contact

Damiano Zanotto: dzanotto@stevens.edu
Jacqueline Montes: jm598@cumc.columbia.edu

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ACADEMIC PROFILE

Institution

SUNY at Buffalo

University at Buffalo The State University of New York
Jacobs School of Medicine and Biomedical Sciences

Indication

DMD and Cardiomyopathy

Stage

Preclinical

Description of Therapeutic

GsMTx4 is a peptide inhibitor of a class of ion channels sensitive to mechanical stress. We have developed a synthetic form (GsMTx4-D) made from digestion-resistant D-amino acids. Mechano-sensitive channels come primarily from a new protein family called Piezo. These channels have recently been intensely studied for their role in many pathologies where tissue mechanical dysfunction is a primary etiological agent like cardiovascular disease and inflammation. DMD's main defect is the inability to control mechanical stress. We have demonstrated that GsMTx4-D protects dystrophic skeletal muscle and increases muscle mass.

DMD treatment has reached a transition point focusing on genetic cures rather than alleviating symptoms. As these genetic therapies enter clinical trials, it has become apparent that they are not as potent as originally demonstrated in animal models. Co-therapeutics are needed for these gene therapies to realize their potential. Recent studies show that muscle relaxants targeting ryanodine receptors (RyR) can boost the potency of exon skipping in DMD models by inhibiting elevated Ca2+. But long-term use of these drugs can pose detrimental side-effects. Piezo ion channels are considered a primary contributor to the Ca2+ leak in DMD muscles. Our goal is to show that targeting these channels with GsMTx4-D will reduce RyR leak and increase gene therapy interventions, while protecting muscle from acute injury.

Description of Platform

We have obtained human DMD muscle explant cell lines from our collaborator Dr. Carrie Miceli at UCLA. These cell lines can be used to evaluate adjuvants for gene therapies in vitro. We also have a collaboration with Dr. Christopher Ward of University of Maryland and his company Myologica that will aide us in the preclinical animal studies to demonstrate boosting of exon skipping therapies.

Key References:

- Suchyna, T.M., et al., Bilayer-dependent inhibition of mechanosensitive channels by neuroactive peptide enantiomers. Nature, 2004. 430(6996): p. 235-240
- Ward, C.W., et al., GsMTx4-D provides protection to the D2. mdx mouse. Neuromuscular Disorders, 2018. 28(10): p. 868-877
- Yeung, E.W., et al., Effects of stretch-activated channel blockers on [Ca2+]i and muscle damage in the mdx mouse. J.Physiol, 2005. 562(Pt 2): p. 367-380
- Wang, J., et al., GsMTx4-D is a cardioprotectant against myocardial infarction during ischemia and reperfusion. Journal of Molecular and Cellular Cardiology, 2016. 98: p. 83-94
- Barthélémy, F., et al., Targeting RyR Activity Boosts Antisense Exon 44 and 45 Skipping in Human DMD Skeletal or Cardiac Muscle Culture Models. Molecular Therapy-Nucleic Acids, 2019. 18: p. 580-589.

IP Status

Patent No: US 7,125,847, Oct 24, 2006 - GsMTx4 composition of matter
Patent No: US 7,259,145 B2, Aug. 21, 2007 - GsMTx4-D composition of matter
Orphan Drug Status for GsMTx4 and GsMTx4-D to treat DMD: Designation # 10-3157. Orphaned drug status provides three main advantages to companies developing drugs with the designation:

1)50% tax credit on all expenditures incurred during clinical testing phase
2)Special grants available for clinical testing phase
3)7 years market exclusivity at the time of FDA approval preventing other drug companies from marketing a competitor drug for the same indication

Goals for Presentation

The goal of this presentation will be to describe the current status of GsMTx4-D preclinical trials demonstrating cardio and skeletal muscle protection. We will present evidence suggesting Piezo channel targeting as a potentially important intervention to improve skeletal and cardiac muscle Ca2+ balance, increase muscle cell output, and boost genetic therapies to repair the dysfunctional protein. We will describe our research plan to produce preliminary data that will be used as the basis for an NIH proposal or to attract investors interested in funding continuing research into this new treatment strategy.

Presenter Name

Thomas Suchyna

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Timestamp: Day 2 32:02 – 46:35

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ACADEMIC PROFILE

Institution

University of California Berkeley

Berkeley UNIVERSITY OF CALIFORNIA

Indication

Measurement of muscle mass

Stage

Clinical

Description of Therapeutic

The D_3creatine dilution test is the first, non-invasive, accurate method to measure functional muscle mass. We are currently funded by MDA, PPMD, and DUK to measure longitudinal changes in muscle mass in boys with DMD as an index of disease progression. The loss of skeletal muscle mass is a common feature of aging, cachexia, and muscular dystrophy. Until now, there has been no method to measure muscle mass in humans. Using this method, we reported that in older men, muscle mass (but not lean body mass) is strongly associated with risk of disability, falls, fractures and mortality. This method is validated in adults, infants, and children. Changes in functional muscle mass in boys with DMD may be a biomarker of disease progression therapeutic efficacy.

Description of Platform

Subjects/patient ingest a single oral, tracer dose of D_3creatine. A single, spot urine sample collected two days later is analyzed for D_3creatinine enrichment by mass spectrometry. Because ~98% of total body creatine is found in muscle (co-located with contractile proteins), this is a measurement of functional muscle mass. Creatine is undiluted by fibrosis and lipid in muscle. We have recently shown that muscle mass in boys with DMD (age 6 - 17) is substantially lower that that seen in healthy age-matched control. In non-ambulant boys, muscle mass was < 5% of body weight.

IP Status

We have three granted patents, US, Canada, and Europe.

Goals for Presentation

To interest investors in establishing a newco with the inventors (William Evans and Marc Hellerstein both UC Berkeley faculty) to commercialize this method for neuromuscular disease, cachexia, pediatrics, aging.
Research: We currently have > 10 funded grants to examine muscle mass in clinical populations including DMD
Medical diagnostic test: The FDA recognizes muscle wasting and sarcopenia (ICD 10 code: M62.84), the diagnostic criteria for these syndromes have not been approved. At the present time there is no clinical test for muscle mass. We will establish the value and even necessity of the D3Cr test for diagnosis of sarcopenia, cachexia, muscular dystrophies and other muscle-related medical conditions and for monitoring the effects new muscle therapies.

William J. Evans

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Timestamp: Day 2 12:25 – 32:02

MDA 2021 CLINICAL & SCIENTIFIC CONFERENCE
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ACADEMIC PROFILE

Institution

University of California, Irvine

Indication

DMD, Skeletal Muscle Regeneration, Support of Muscle Stem Cells, Neuromuscular Diseases, Development defects

Stage

Preclinical

Description of Therapeutic

Cell based therapy using human-iPSC generated skeletal muscle cells for muscle wasting diseases and personalized medicine

Description of Platform

HPSCs are a powerful tool for studying muscle regeneration and stem cell niche development. The ability to establish new muscle and niches is important for effective long-term cell therapies, in which transplanted muscle stem cells must balance the formation of new muscle fibers as well as maintain the stem cell pool. We have developed a robust approach to differentiating hPSCs to skeletal muscle for transplantation. We have demonstrated hPSC SMPCs fuse to form hundreds of new myofibers in vivo. Evaluating the regulators of skeletal muscle formation during regeneration and development will improve our ability to generate *de novo* human niches and better support human PAX7 cells and muscle function *in vivo* for cell and regenerative therapies.

IP Status

PCT/US 62/443,499. Title: Methods for generating skeletal muscle cells from human pluripotent stem cells.

Goals for Presentation

Highlight new perspectives on muscle cell based therapies. Receive funding for research directions and assistance with translating cell based therapies for muscle wasting diseases.

Presenter Name

Michael Hicks

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Timestamp: Day 1 8:28 – 29:00

ACADEMIC PROFILE

Mitochondrial Disease

University of Granada

UNIVERSIDAD DE GRANADA

Indication

Mitochondrial Disease, Obesity

Stage

Preclinical

Description of Therapeutic

ONE MOLECULE, TWO THERAPEUTICS APPLICATIONS

1) Treatment of a rare mitochondrial disease (`possibility of Orphan Drug designation) - https://pubmed.ncbi.nlm.nih.gov/30482867/.

2) Prevention and treatment of overweight and obesity (a patent has been filed) - https://www.biorxiv.org/content/10.1101/2021.04.13.438670v1.

Description of Platform

NA

IP Status

A patent was filed on December 2020.

Goals for Presentation

We are looking for partners and investors for further development of the therapeutic options.

Presenter Name

Luis C. López, PhD
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Timestamp: Day 1 1:07:00 – 1:21:24

MDA 2021 CLINICAL & SCIENTIFIC CONFERENCE
Insights in Research Investor Summit

ACADEMIC PROFILE

Institution

University of Massachusetts Medical School

University of Massachusetts Medical School

Indication

FSHD: Facioscapulohumeral Muscular Dystrophy

Stage

Preclinical

Description of Therapeutic

DUX4 is a transcription factor that is only active at early embryonic stages and in testes. Tandem copies of DUX4 are packaged in repetitive DNA sequences called D4Z4. The general population has between 11 to 150 D4Z4 repeats which cause epigenetic DUX4 silencing. The contraction of the D4Z4 in FSHD1 patients to 10 repeats or less causes inefficient DUX4 silencing. DUX4 in turn activates the transcription of numerous downstream targets, eventually causing cell death and muscle wasting. Here we suggest the use of siRNA to target DUX4 and reduce its expression. This should also reduce DUX4 downstream targets and thus stop or slow down muscle wasting. To achieve this, we created a proprietary siRNA muscle targeting platform. In addition, we developed a xenograft mouse model to test DUX4 reduction in FSHD patient myoblasts in vivo. Here we show that our siRNA (DU01) reduces DUX4 and DUX4 biomarkers expression both *in vitro* in patient-derived cells and *in vivo* in the xenograft mouse model.

Description of Platform

While siRNA could be a potential treatment for several diseases, siRNA delivery to desired organs remains challenging at best. Liver targeting has been the "low hanging fruit" due to the unique functions of the liver, however, there is a huge unmet need for extrahepatic delivery. UMMS has been a leader in the field through creating and validating a CNS targeting platform in non-human primates. This platform led to the creation of Atalanta therapeutics, a new biotech company focused on neurodegenerative diseases and has, to date, raised $110 million. Here we showcase the creation of a new siRNA muscle delivery platform based on the discovery of a new conjugate (DCA) that allows for significant skeletal muscle and heart accumulation. This platform could address a significant unmet need in the field and would allow the targeting of many muscular dystrophies (including but not limited to FSHD), cancer muscle wasting, and acute muscle injury.

IP Status

We have multiple filed IP on backbone modification, linker, and DCA conjugate for muscle targeting. We are currently in the process of filing new IP to protect DU01 and other siRNA sequences.

Goals for Presentation

We are seeking a partner to help us further develop the FSHD asset and the muscle delivery platform. This could take multiple forms whether through sponsored research agreements to develop new targets, licensing the FSHD asset and/or the muscle delivery platform, or NewCo formation around the muscle delivery platform.

Presenter Name

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ACADEMIC PROFILE

MDA CLINICAL & SCIENTIFIC CONFERENCE 2021
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Institution

University of Minnesota

Indication

Ambulatory Breathing Monitor

Stage

Preclinical

Description of Therapeutic

Estimation of three dimensional thoracoabdominal displacements during respiration using inertial measurement units. Accepted for publication, IEEE/ASME Transactions on Mechatronics

Description of Platform

This project refines a wearable sensor that can measure chest wall kinematics during breathing by sensing forward, upward, and circumferential motion of the abdomino-thoracic compartment with each breath. Time-domain relationships among movement of the abdomen, lower ribcage, and upper ribcage, are estimated to describe how function of respiratory muscles responsible for movement of these structures is altered during disease evolution. The device is designed to provide ambulatory as well as in-hospital respiratory health monitoring. Changes in breathing pattern can be tracked over time, and trends identified, thereby allowing early recognition of clinical deterioration. Conversely, as the respiratory system improves, the breathing pattern reverts predictably and gradually towards normality and can also be tracked. Objective and valid outcome measures of respiratory function are also needed to test treatment efficacy in clinical trials of new therapies. Optoelectronic plethysmography has already been used to study breathing kinematics in infants with spinal muscular atrophy in the laboratory. Assessing inspiratory muscle strength for early detection of respiratory failure in ALS still relies on crude assessments such as FVC and maximal inspiratory pressure (MIP). Between these age bookends are patients with Duchenne MD, in whom recommendations for respiratory monitoring consist of annual FVC while ambulatory, then semi-annual FVC, MIP, SpO_2 and non-invasive PCO_2 checks.

IP Status

PROVISIONAL PATENT APPLICATION: DBC File No. U639.151.101
Filed: Attorney Docket Number U639.151.101.
UM Case No. 2020-338

Goals for Presentation

We are completing studies in healthy volunteers during loaded breathing, stressing the respiratory muscles to induce thoraco-abdominal movement asynchrony, mimicking displacements seen on torso during failure of respiratory muscles. We are applying for research grants to continue this research, specifically:
Specific Aim 1: Development and evaluation of machine learning algorithms for automatic recognition of normal breathing or stressed breathing indicated by abdomino-thoracic lag, or abdomino-thoracic paradox, with wearable sensors (work in progress). Phase-angles differentiating normal from loaded breathing conditions will be computed.
Specific Aim 2: Refinement of wearable sensor system in preparation for clinical use, including device packaging, development of device self-diagnostics, software user interface, fabrication of adequate numbers of devices, and verification of all devices on human subjects.
• Maximum of 6 devices per subject: 2 devices affixed on upper rib cage, 2 on lower rib cage and 2 on abdomen. Miniaturize them for use during infancy.
• Displaying real-time data graphics of tidal volume (VT), from 3 sets of time-synchronized displacements (upper ribs, lower ribs and abdomen), and inference on the type of breathing (normal, abdomino-thoracic lag, abdomino-thoracic paradox).
We are requesting presentation to this audience to solicit partnership(s) for research, development, and refinement.

Presenter Name

Paolo Pianosi
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Timestamp: Day 1 29:00 – 34:30
56:12 – 1:06:30

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ACADEMIC PROFILE

Institution

University of Padova, Department of Biomedical Sciences

Indication

Sarcoglycanopathies

Stage

Preclinical

Description of Therapeutic

We propose a pharmacological approach to treat sarcoglycanopathy caused by missense mutations. The pathogenic mechanism of these forms of sarcoglycanopathy is similar to the one of cystic fibrosis, in which a folding defective protein is prematurely degraded leading to a loss of function.
We argued that the small molecules known to correct a defective CFTR, could also be of benefit in sarcoglycanopathies.
The POC of such pharmacological approach was established in vitro, and recently in vivo.
We are evaluating efficacy of the therapeutic strategy in additional diseases sharing similar pathogenic mechanism.

Description of Platform

N/A

Goals for Presentation

We are looking for partners interested in funding/collaborating for concluding the preclinical steps of the drug development pipeline and for collecting the requirements for applying for ODD in EU and US. All these activities are preliminary to the design of the subsequent clinical trials.

IP Status

IP1) A CFTR corrector for the treatment of genetic disorders affecting striated muscle
OWNERSHIP: University of Padova
INVENTORS: D. Sandonà, R. Sacchetto, E. Bianchini, P. Volpe, F. Mascarello, R. Betto
EP 2925317 granted (2019) validated in IT, SE, BE, UK, CH, NL, DE, FR, DK, IE
US 9,987,256 B2 granted (2018)
US continuation 15/945854 filed 04.05.2018 pending
Italian patent 0001414647 granted (2015)

IP2) Combination Treatment of Sarcoglycanopathies
OWNERSHIP: University of Padova; INSERM, Genethon, Université d'Evry
INVENTORS: D. Sandonà, I. Richard
Patent pending in:
EU 3737375; China 20198000821.3; Canada 3,088,229; Japan 2020-559019; US 16/960,503 Hong Kong 62020021527.0

Presenter Name

Dorianna Sandona

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ACADEMIC PROFILE

MDA CLINICAL & SCIENTIFIC CONFERENCE 2021
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Institution

University of Pittsburgh

Indication

Lambert-Eaton myasthenic syndrome, SMA, Congenital myasthenic syndromes, ALS, normal age-induced frailty in the elderly, botulinum toxin A poisoning

Stage

Preclinical

Description of Therapeutic

We have discovered novel voltage-gated calcium channel positive allosteric gating modifiers that increase calcium entry at the neuromuscular junction during action potential activity and are selective for the Cav2 family of calcium channels. This is the result of an ongoing collaboration between Drs. Peter Wipf (Chemistry Dept., Univ. Pitt.) and Stephen Meriney (Neuroscience Dept., Univ. Pitt.). We initially identified "GV-58", a novel analog of (R)-roscovitine (Liang et al., 2012, Synthesis and Biological Evaluation of a Selective N- and P/Q-Type Calcium Channel Agonist. ACS Medicinal Chemistry Letters, 3(12): 985-990), and have now generated and analyzed over 200 related analogs (Wu et al., 2018, New Cav2 calcium channel gating modifiers with agonist activity and therapeutic potential to treat neuromuscular disease. Neuropharmacology, 131: 176-189). Our novel gating modifiers (GV-58 and analogs thereof) do not directly open calcium channels, but instead prolong the mean open-time of calcium channels that have been induced to open by depolarization. This property underlies the synergistic effects of these novel gating modifiers with 3,4-diaminopyridine (Tarr et al., 2014, Complete reversal of Lambert-Eaton myasthenic syndrome synaptic impairment by the combined use of a K+ channel blocker and a Ca2+ channel agonist. J Physiol, 592(16): 3687-96). Therefore, our new calcium channel gating modifiers could be used alone, or in combination with 3,4-diaminopyridine.

Description of Platform

Our new leads generated by strategic design and synthesis (by Dr. Peter Wipf's group in the Chemistry Department at the University of Pittsburgh) are screened using patch clamp electrophysiology in cultured HEK293 cells expressing Human Cav2.1 channels and functional testing in mouse models of neuromuscular diseases using ex vivo electrophysiology of neuromuscular function and behavioral tests of muscle strength.

IP Status

GV-58 and closely related analogs have been patented in the US (US Patents issued: No. 9,796,714 (October 24, 2017); 10,174,031 (January 8, 2019); 10,752,629 (August 25, 2020). The goal is to identity an industrial partner to assist in (1) using our knowledge of the structure-activity relationship of these novel gating modifiers to strategic synthesize and scale-up new optimized therapeutic leads (with new IP potential), (2) testing the potency and efficacy of new leads, and (3) ADME-Tox studies of the most promising lead compounds. The goal is to develop a pre-clinical package that will allow us to advance a new clinical candidate molecule.

Goals for Presentation

Our goal is to present the background on the development of our novel Cav2 calcium channel gating modifiers and their evaluation using in vitro, ex vivo, and in vivo models of neuromuscular disease and dysfunction. In addition we would present our development plan to refine this chemotype for further pre-clinical development and identify a clinical candidate with new international IP protection. We have demonstrated that we can generate novel analogs of GV-58 with improved properties (and independent IP) and we would plan to develop a new analog with 5x higher potency and stronger effects on neurotransmitter release for in vivo applications. This new lead analog would also maintain or improve on the preclinical pharmacology, PK properties, and safety profile obtained with GV-58.

Presenter Name

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View Presentation and Recording
Timestamp: Day 2 1:15:15 – 1:28:00

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ACADEMIC PROFILE

Institution

University of Western Australia

THE UNIVERSITY OF WESTERN AUSTRALIA

Indication

Screening biomarkers for DMD treatments

Stage

Preclinical

Description of Platform

NA

Description of Therapeutic

One of the many challenges in developing or modifying therapies for DMD is that long treatment times (about 12 months) are required before a meaningful functional clinical outcome can be obtained. Consequently, clinical trials are expensive, resource-intensive and time consuming.

Molecular biomarkers that could respond relatively rapidly (within days or weeks) to putative treatments for DMD would be highly desirable screening tools before undertaking a lengthy clinical trial. We have developed a screening tool, an at-home collection device, to measure novel responsive protein biomarkers.

IP Status

Confidential

Goals for Presentation

Our goal is to link with companies interested in:

(1) Further developing and validating the technology in partnership

(2) Licensing

Presenter Name

Peter Arthur

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Timestamp: Day 2 1:28:40 – 1:45:10

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COMPANY PROFILE

Company

AavantiBio, Inc.

AAVANTIBIO

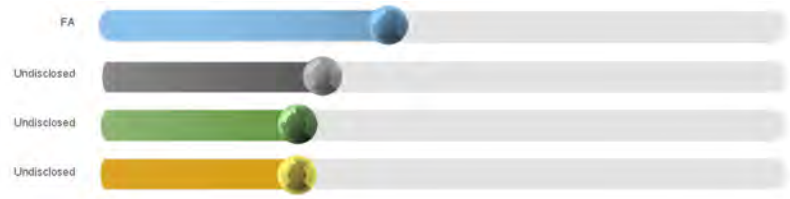
Indication(s)

Friedreich's Ataxia

Stage

Preclinical

Pipeline



Therapeutic Platform

Gene therapy utilizing AAV vectors

Management Team

Bo Cumbo; President and CEO
Douglas Swirsky; Chief Financial Officer
Ty Howton, COO and General Counsel
Christopher Wright, M.D., Ph.D.; Chief Medical Officer
Paul Herzich; Chief Technology Officer

Board and Other Advisors

Barry Byrne, M.D., Ph.D.
Manuela Corti, PT, Ph.D.
Bo Cumbo
Ellen Hukkelhoven, Ph.D.
Ian F. Smith
Benjamin Lund
Jake Simson, Ph.D.

Presenter Name

Barry Byrne, MD, PhD
Founder and Director,

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Timestamp: Day 2 3:09:14 – 3:28:10

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COMPANY PROFILE

Company

AAVogen Inc.

AAVogen

Indication(s)

Inclusion body myositis (lead), DMD (developing)

Stage

Preclinical

Pipeline

Developing treatments for muscle wasting have been weakly effective or created serious safety concerns. Our solution is AVGN7; a novel gene therapeutic for attenuating processes that normally inhibit muscle growth. This includes ActRIIb ligands and inflammatory cytokines. By "inhibiting inhibition", muscle growth and function are rapidly enhanced and muscle wasting is prevented. Moreover, the technology is muscle specific and attenuates a multitude of inhibitory signals. This prevents off-target effects while enhancing efficacy.

Therapeutic Platform

AVGN7 (rAAV6:Smad7)

- Gene therapeutic
- Recombinant adeno-associated virus, serotype 6 capsid
- Human Smad7 cDNA "payload" gene
- Proprietary promoter/regulatory cassette constructed from the muscle creatine kinase promoter

Management Team

Dan Rodgers, PhD; Founder & CEO
William Mann, PhD; Operations & Development
Heather Webb-Hsu, PhD; Project Management
Jade Brown, MBA; Business Development & Licensing
Carole Bellis, Partner, DLA Piper; Legal
Tim Gehring, CPA; Finance
Peter Korytko, PhD; Preclinical Development
Jeff Fellows; Regulatory Affairs

Board and Other Advisors

SCIENCE ADVISORY BOARD:
Lawson Macartney, DVM/PhD; Ambrx
Paul Gregorevic, PhD; University of Melbourne
Jeff Chamberlain, PhD; University of Washington
Charles Murry, MD; University of Washington
Denis Guttridge, PhD; MUSC

Presenter Name

Dan Rodgers, PhD
Founder & Chief Executive Officer

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COMPANY PROFILE

MDA 2021 CLINICAL & SCIENTIFIC CONFERENCE
Insights in Research Investor Summit

Company

Abilitech Medical, Inc.

abilitech MEDICAL

Indication(s)

DMD, ALS, BMD, LGMD, FSHD, SMA, MS

Stage

Clinical

Pipeline

2021 Abilitech™ Assist launch

2022 Abilitech™ grip device

2024 Abilitech™ Ambulatory device

Therapeutic Platform

Our device assists and supports a non-functioning arm to support activities of daily living like eating, opening doors, and using a computer. We require a minimal amount of strength such as a manual muscle test of a 2- or greater in the shoulder and elbow to gain full use of our device. We believe and intend to study that there may be the opportunity to build strength and limit compensation, which can cause painful side effects.

Management Team

Angie Zavoral Conley, CEO and President
Nancy Ness, CFO
Clare Padgett, VP of Research and Development
Jason Graves, VP of Sales
Lisa Ramme Latterell, Contract Marketing
Shawna Persaud, Ph.D. Director of Clinical and Product Management
Kevin Symms, Director of Reimbursement
Ryan Bauer, Senior Mechanical Engineer
Mark Orseschnick, Director of Sustaining and Continuation Engineering
Eli Krumholz, Ph.D. Director of Software Development.

Board and Other Advisors

James Ehlen, M.D. Abilitech Medical board chair and Independent
Aaron Fletcher PhD, Bios Partners
Elona Baum, Managing Director, DEFTA Partners.
Angie Zavoral Conley, Abilitech Medical Founder, President and CEO.

Scientific Advisors:
Dr. Mark Gormley, Physiatrist
Dr. Peter Karanchunski, Neurologist

Presenter Name

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Timestamp: Day 1 3:27:00 – 3:42:45

COMPANY PROFILE

Company

Aquilus Pharmaceuticals

Indication(s)

ALS

Stage

Late Preclinical

Pipeline

1) A small molecule, orally bioavailable, MMP inhibitor for the treatment of Amyotrophic Lateral Sclerosis (ALS)- Late preclinical stage.

Therapeutic Platform

Aquilus Pharmaceuticals (Aquilus) has a portfolio of very potent & selective

matrix metalloproteinase (MMP) inhibitors for treating various inflammatory and CNS disorders

For a copy of the slide presentation please e-mail me at: sucholeiki@aquiluspharma.com

Management Team

Irving Sucholeiki, Ph.D., President & CEO

Darrell J. Nix, Ph.D., Vice President of R & D

Dr. Roy Sucholeiki, M.D. Vice President of Clinical Development

Board and/or Other Advisors

Rita Sattler, PhD. Associate Professor, Neurobiology Barrow Neurological Institute St. Joseph's Hospital and Medical (consultant).

Daniela Zarnescu, Ph.D., Professor of Molecular and Cellular Biology, University of Arizona (Consultant).

Presenter Name

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Timestamp: Day 2 3:47:10 – 3:59:45

COMPANY PROFILE

Company

Constant Therapeutics

Indication(s)

DMD, Cardiomyopathy, other muscular dystrophies

Stage

Active IND

Pipeline

TXA127: COVID-19 Phase 2; Stroke recovery Phase 2; DMD Cardiomyopathy Phase 2

NMEs: Preclinical

Therapeutic Platform

COVID-19; inflammation; fibrosis

Neurology; Neuromuscular; Neurodegenerative

Orphan Diseases

Management Team

Rick Franklin, President & CEO
Elizabeth Wagner, COO

Board and Other Advisors

Joerg Gruber
Alex Shlyankovich
Thomas Voit

Presenter Name

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Timestamp: Day 2 3:28:14 – 3:47:10

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COMPANY PROFILE

Company

Enable Therapeutics

Indication(s)

Glycogen Storage Diseases, Myofibrillar Myopathy, Myotubular Myopathy, Duchenne Muscular Dystrophy

Stage

Early clinical to Preclinical

Pipeline

Fab-GAA (Pompe [phase 1/2 completed], Polyglucosan Diseases [IND submission 2Q22], Fab-AMY (Fab-GAA alternative); Fab-NEP (Myofibrillar Myopathy [awarded phase 1 STTR]); Fab-microdystrophin (Duchenne Muscular Dystrophy [preclinical]); Fab-BIN1 (Myotubular Myopathy [awarded phase 1 SBIR])

Therapeutic Platform

The Enable Therapeutics platform is based on a muscle- and CNS-penetrating antibody that can deliver full-length proteins and oligonucleotides to the cytoplasm. This antibody was first isolated from a mouse model of Systemic Lupus Erythematosus. We then humanized and optimized 3E10 for more robust cellular uptake. The Fab fragment of 3E10 has been shown to be capable of delivering full-length protein cargo into cells in many ways. Internalization requires Equilibrative Nucleoside Transporter 2 (ENT2; SLC29A2), which is particularly enriched in human and murine skeletal and cardiac muscle; ENT1 and other ENT isotypes do not facilitate uptake. The Fab fragment of 3E10 fused to recombinant human acid-alpha glucosidase (rhGAA) substantially improves skeletal and cardiac biodistribution compared to rhGAA alone, in a series of radiolabel- and ELISA-based biodistribution studies in mice. Fab-GAA demonstrated promise in a 12-patient Phase 1/2 clinical trial in late-onset Pompe Disease, with a clean safety profile similar to rhGAA (Lumizyme, Sanofi-Genzyme) standard of care and trends of improved or stable disease in patients previously on Lumizyme (NCT02898753). Our Fab-based platform has been validated in several additional ways including facilitating cellular penetration of active, otherwise non-cell penetrating enzymes (e.g., AMY, NEP, MTM, MBNL1).

Management Team

Robert Shaffer, Ph.D. – Founder, CEO
Dustin Armstrong, Ph.D. – Founder, President/CSO

Board and Other Advisors

Enable Therapeutics is a new entity having been incorporated only several months ago. We have ongoing academic collaborations with expert researchers who bring unique knowledge to the table.

Presenter Name

Robert Shaffer
Founder/CEO

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Timestamp: Day 2 3:59:50 – 4:15:45

COMPANY PROFILE

Company

Encefa

Indication(s)

ALS, PD, AD, HD, MS, DMD

Stage

Preclinical

Pipeline

NC-B8, a First-in-Class humanized, brain penetrant, potent and selective IgG4-S228P mAb, generated from a synthetic bank of humanized phages. 18 months away from IND/ IMPD filing (clonal selection achieved). Cross-reactive (mice, rats, cynomolgus and dogs). Strong potent preclinical POC of efficacy (5 different mice models, hPBMCs, old dogs spontaneously affected by an ALS-like disease), good safety profile, intravenous administration.

Therapeutic Platform

First-in-Class drugs against a wide range of neuro-muscular and neuro-degenerative diseases, by activating directly both the Autophagy-Lysosomal Flux (up to lysosomal exocytosis), and the Energy Metabolism (insulin-independent glucose uptake) of CD38+ cells (NAD-independent pathway).

CD38 is an age-related target known to be a marker of pro-inflammation and linked to senescence mechanisms. Novel approach on CD38, epitope-related (3 patents filed). Differentiation from existing CD38-compounds strongly established. Companion biomarkers for precision medicine.

Management Team

Laurence Bressac, Co-Founder and CEO
Damien Toulorge, PhD, Co-Founder and CSO
Serge Guerreiro, Co-Founder and Chief Translational Officer

Board and Other Advisors

Pr. Pierre-François PRADAT, MD, PhD, Neurologist at hôpital de la Pitié Salpêtrière, clinician specialized in ALS
Pr. Serge PRZEDBORSKI, MD, PhD, Neuroscientist, Vice Chair of Research at Columbia University,
Dr. Etienne HIRSCH, PhD, Neuroscientist, Director of the neurosciences at Aviesan and INSERM

Presenter Name

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COMPANY PROFILE

MDA 2021 CLINICAL & SCIENTIFIC CONFERENCE
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Company

Esperare

Stage

Phase II Clinical Trial

Indication(s)

Cardiomyopathy of DMD

Pipeline

The sodium proton exchanger type-1 (NHE-1) inhibitor, Rimeporide, represents an innovative and promising target for Duchenne muscular dystrophy (DMD) patients. The involvement of NHE-1 in cardiac pathology, including cardiomyopathy, has been described for more than a decade in the literature. Recent studies in mdx mice, GRMD dogs and cardiomyopathic hamsters support the use of rimeporide as a cardioprotective agent. Overall, Rimeporide's data package indicates that it can prevent inflammation and the long-term accumulation of fibrosis in dystrophic muscles, the hallmarks of progressive disease. Rimeporide has also be shown to be safe and well tolerated in healthy adults. Given the lack of really effective therapeutic options for dystrophic cardiomyopathy in patients with Duchenne and given that cardiomyopathy is the leading cause of premature death in those patients, the effects of rimeporide on the heart observed in several relevant animal models (mdx mice and cardiomyopathic hamster) provide a positive indication that early intervention with a disease mechanism-specific cardioprotective agent such as rimeporide could bring about a long-term clinical benefit.

Therapeutic Platform

Our focus is to translate research findings into new therapies for rare diseases through drug (re)positioning. EspeRare concentrates its efforts on rare diseases that have the highest unmet medical needs and for which there is sufficient scientific understanding. Based on collaboration with pharmaceutical companies, biotechs and academia, the foundation builds a pipeline of therapies to be (re)positioned for rare diseases. We work with established patient networks and biomedical centres of excellence to validate the therapeutic opportunities from preclinical to early clinical stages. Once proof of concept in humans is validated and a conclusive data package is generated, compounds can go back to the originator or be transferred to biomedical partners for later stage clinical trials, registration and commercialisation.

Management Team

Caroline Kant, Founder & CEO
Florence Porte Thomé, Founder & CSO
Dr Julian Gray, Chief Medical Officer
Dr Sameera Allie, Medical Director

Board and Other Advisors

Cardiologists
Pr K. Hor
Pr J. Soslow
Pr C. Spurney
Pr : J. Bourke

Neuropediatricians
Pr F. Muntoni
Dr S.Previtali
Dr L. Servais

Board
Sharon Terry: President of EspeRare, Board President of Genetic Alliance (USA)
Béatrice Greco: Founder and Board Member
Peter Potter-Lesage: Board Member and Treasurer
Ewen Sedman: Board Member
Denis Mortier: Board Member & Chair of the Business Advisory Committee

Presenter Name

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View Presentation and Recording
Timestamp: Day 2 3:59:50 – 4:15:45

MDA CLINICAL & SCIENTIFIC CONFERENCE 2021
Insights in Research Investor Summit

COMPANY PROFILE

Company

Extrave Bioscience, LLC

Indication(s)

Duchenne muscular dystrophy, Becker muscular dystrophy, several LGMDs

Stage

Preclinical

Pipeline

Our DMD/BMD therapeutic is our most advanced asset. We have demonstrated that we can effectively package the full length dystrophin protein and transcript inside extracellular vesicles. Our vesicles effectively delivered dystrophin to muscle fibers and dystrophin was correctly localized to the sarcolemma within the fibers. Further, dystrophin delivery allowed restoration of the dystrophin-glycoprotein complex indicating dystrophin is functional. Finally, these effects persisted for at least three weeks following a single injection. We are able to target a host of other neuromuscular diseases using a similar approach. We are also able to package conventional AAV, which may improve the efficacy of gene therapy.

Therapeutic Platform

We have discovered how to harness endogenous cellular machinery to produce extracellular vesicles containing our cargo of interest using a highly scalable method without the need to manipulate production-cell genetics. Because of this approach we are able to produce vesicles that contain the full-length dystrophin protein and transcript without independent production, isolation, and packaging of either. We are also able to package a host of other proteins and transcripts that would be therapeutically useful for a number of indications of interest to the MDA. Our platform can also be readily applied to gene therapy production and delivery of AAVs with the promise of increased production efficiency, increased delivery efficiency, and immune evasion, which may facilitate treatment of patients with existing neutralizing antibodies as well as redosing with AAV should it be needed. Finally, with minor modification to production cells, we are able to package siRNA and miRNA for therapeutic use.

Management Team

Matthew Hudson, PhD
Joshua Selsby, PhD
Brittany Wilson, PhD

Board and Other Advisors

TBD

Presenter Name

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Co-Founder

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View Presentation and Recording
Timestamp: Day 1 5:42:25 – 6:03:35

COMPANY PROFILE

Company

Ixchel Pharma

Indication(s)

Friedreich's ataxia, Leigh Syndrome, Mito-myopathy

Stage

Preclinical

Pipeline

IXC-109

Therapeutic Platform

IXC-109 is a small-molecule drug with the potential to treat multiple orphan mitochondrial diseases. Through increasing mitochondrial biogenesis, Ixchel's Novel Chemical Entity (NCE) IXC-109 is the first small molecule to rescue Friedreich's ataxia and Leigh Syndrom animal models. IXC-109 outperforms other marketed drugs of a similar chemical category when tested head-to-head. IXC-109 releases two molecules that have each been previously dosed in millions of humans, presaging an accelerated regulatory path with the potential for Priority Review Vouchers (PRVs). Ixchel has an experienced Science, Pharmacology and Regulatory and Clinical Team with over 100 years aggregate experience.

Management Team

Gino Cortopassi, PhD, CEO
Abhinav Dhandia, COO
Paul Maffuid, PhD, Regulatory Affairs
Zane Starkewolfe, PhD, CBO
Susan Perlman, MD, Clinical Adviser
Somdutta Sen, Pharmacology & Pharmacokinetics
Alexey Tomilov, PhD, Pharmacologist

Board and Other Advisors

Gino Cortopassi
Abhinav Dhandia
Zane Starkewolfe

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Timestamp: Day 2 4:15:52 – 4:40:20

MDA CLINICAL & SCIENTIFIC CONFERENCE 2021
Insights in Research Investor Summit

COMPANY PROFILE

Company

Juvena Therapeutics

Juvena THERAPEUTICS

Indication(s)

Biologic - muscle regeneration

Stage

Preclinical

Pipeline

Juvena is developing 3 muscle regenerative protein therapeutic candidates in lead optimization for muscular dystrophy, muscle wasting, and acute injury-related indications. Juvena is raising a Series A to advance lead program through IND and Phase 1/2 clinical studies for Myotonic Dystrophy Type 1 (DM1).

Therapeutic Platform

Juvena Therapeutics is a regenerative medicine startup developing breakthrough protein therapeutics derived from human embryonic stem cells (hESCs) for degenerative diseases using state-of-the-art proteomics and machine learning (ML). Juvena's ML-enhanced drug discovery and preclinical development platform identifies lead therapeutic candidates from a proprietary pro-regenerative, (hESC)-derived protein library to create tissue-specific medicines for degenerative and rare diseases. Initial therapeutic programs are advancing leads for muscular dystrophies, atrophy, and injury. Since Juvena's founding in 2017, Juvena has discovered several regenerative protein therapeutic candidates with strong human *in vitro* and mice *in vivo* efficacy and established multiple issued and patent-pending composition of matter formulations. Juvena is advancing a lead program for Myotonic Dystrophy Type 1, a rare autosomal dominant, progressive muscle-wasting disease. Juvena Therapeutics is raising a Series A to develop their top lead to a clinical-stage investigational new drug and leverage their discovery platform to rapidly identify and validate new protein drug candidates for tissue-specific degenerative diseases. Please visit their website to learn about Juvena's science and technology and their Meet the Team Page to learn more.

Management Team

Juvena was founded on a decade of scientific discoveries in aging biology, tissue degeneration, and proteomics by Hanadie Yousef, PhD, CEO and Jeremy O'Connell, PhD, CSO.

Board and Other Advisors

Board of Directors:
Stephen Juelsgaard, DVM, JD; Former EVP at Genentech; Stanford Law
Strategic Advisors:
Mike Nohaile, PhD: Amgen, Novartis, McKinsey, UC Berkeley, MIT
Matthew Fust, MBA: Former CFO Onyx, Perlegen Sciences, ALZA Corporation
Mark Leslie: Stanford GSB, Stanford Healthcare, Veritas
Scientific Advisors:
Professor David Schaffer, PhD: UC Berkeley, Director, Berkeley Stem Cell Center, co-founder, 4D Molecular Therapeutics
Professor Joe Wu, MD, PhD: Stanford School of Medicine, Director, Stanford Cardiovascular Institute
Professor Peter Jackson, PhD: Stanford School of Medicine, former Director, Genentech
Professor Irina Conboy, PhD: Bioengineering, UC Berkeley, expert KOL, tissue rejuvenation and aging biology
Professor Nicholas E. Johnson, MD, MSCI, FAAN: Professor and Vice-Chair of Research, Neuromuscular Diseases, VCU; expert KOL, DM1 & dystrophies

Presenter Name

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CEO and co-Founder

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COMPANY PROFILE

MDA 2021 CLINICAL & SCIENTIFIC CONFERENCE
Insights in Research Investor Summit

Company

Minicircle

Indication(s)

ALS and MD

Stage

Preclinical

Pipeline

We have a in vitro data, a mouse study showing a doubling of muscle mass in a short period of time and we have self-tested our vector showing intens,e mental and physical effects accompanied by sustained follistatin-344 expression levels in the blood for longer than one year. Data is gathered via ELISA.

We are seeking funding and clinical advisors for FDA IND-enabling studies.

Therapeutic Platform

Minicircle has developed a non-inflammatory plasmid vector for follistatin-344, the tissue-specific myostatin-inhibiting hormone. Follistatin decreases chronic inflammation, increases muscle mass, cardiovascular health, and cognitive well-being - enhancing quality of life for people with MD and ALS.

The platform uses a lyophylizable polymer transformation reagent which allows the therapy to be distributed without a cold chain and to have a long shelf life in harsh conditions. We have designed this therapy to be used everywhere in the world - not just to in privileged first world clinics.

Our long-game is to pursue this as a treatment for healthy aging/ longevity.

Management Team

Mac Davis • Founder, CEO biotechnologist and startup entrepreneur

Walter Patterson • Founder, CSO, molecular biologist

Board and Other Advisors

Business advisors:
Robert Rhinehart• founder of Soylent, VC
Sam Altman • Silicon Valley VC
Brian Varnum - Former VP of Amgen

Science advisors:
Corklin Steinhart • Global Director at Merck
Brian Johns• Head of R&D at GlaxoSmithKline

Presenter Name

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Timestamp: Day 2 2:14:28 – 2:33:50

COMPANY PROFILE

p

Company

miRecule Inc.

Indication(s)

FSHD

Stage

Preclinical

Pipeline

MC-DX4 for Facioscapulohumeral Muscular Dystrophy (FSHD)

miRecule is developing MC-DX4 for the treatment of Facioscapulohumeral Muscular Dystrophy (FSHD). MC-DX4 is an Antisense oligonucleotide (ASO) that targets and represses the DUX4 gene. The ASO is conjugated to a muscle specific antibody via miRecule's proprietary Muscle-NAVTM technology to assist in delivery to affected muscle cells throughout the body.

Therapeutic Platform

miRecule's proprietary DREAmiR™ platform utilizes genomic and outcome data from patients to identify underlying genetic target sequences that cause their disease, and then creates a novel RNA therapeutic that can directly target and fix that genetic abnormality. For FSHD, we have identified best-in-class antisense oligonucleotides that are chemically modified for enhanced stability, potency, and safety profiles. To deliver our anti-DUX4 ASO, miRecule is developing a proprietary antibody delivery technology, known as Muscle-NAVTM. Muscle-NAV targets a unique receptor only expressed on skeletal muscle tissue to avoid safety issues related to ASO delivery to non-diseased tissues. Muscle-NAV also utilizes unique conjugation chemistry that aids in longer bio-distribution and RNA therapeutic delivery to the cytoplasm. miRecule is working to validate its Muscle-NAV delivery platform to create an effective therapeutic for FSHD, as well as a broadly applicable technology platform extendable to other muscle diseases and muscular dystrophies.

Management Team

Anthony D. Saleh, PhD, (CEO and Board Member)

Ashwin Kulkarni, MS (COO)

Board and Other Advisors

Rabi Tawil, MD, Clinical Advisor
Yi-Wen Chen, PhD, Scientific Advisor on therapeutic development for muscular dystrophies.
Paul Miller, PhD, Scientific Advisor on nucleic acid chemistry.
Lawrence Vernetti, PhD, Scientific Advisor on Preclinical Toxicity

Business Advisors
Dave Lemus (Consulting CFO and Board member)
Dr. Lisa Beth Ferstenberg, MD (Consulting CMO and Board Member)
Dr. Christine D. Copple, PhD (Board Member)

Presenter Name

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Timestamp: Day 2 2:53:04 – 3:09:12

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COMPANY PROFILE

Company

Myoarete LLC

Indication(s)

DMD

Stage

Preclinical

Pipeline

Our pipeline consists of utrophin upregulation-based MyoAr products for treatment of Duchenne muscular dystrophy (DMD). These have been identified using new platform technologies developed at the founder's laboratory at the University of Pennsylvania, with patents pending, and are being exclusively licensed to MyoArete.

MyoAr Small Molecules are low molecular weight organic compounds with pharmacological properties suitable for drug development. The MyoAr Small Molecules alleviate mRNA repression and increase utrophin expression.

Therapeutic Platform

Utrophin upregulation platform for Duchenne Muscular Dystrophy (DMD) therapy.

Management Team

Professor Tejvir S. Khurana MD, PhD., Founder

Board and Other Advisors

Professor Donna M Huryn, PhD, Advisor

Presenter Name

Tejvir S. Khurana, MD, PhD

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Timestamp: Day 2 1:45:16 – 1:59:00

MDA 2021 CLINICAL & SCIENTIFIC CONFERENCE
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COMPANY PROFILE

Company

MyoGene Bio

MyoGene Bio

Indication(s)

DMD

Stage

Preclinical

Pipeline

MyoGene Bio is dedicated to developing cutting edge therapies for muscle diseases starting with a gene editing therapy for Duchenne muscular dystrophy. Additional gene or stem cell therapies for other muscle diseases to follow.

Therapeutic Platform

Our first approach, MyoDys$^{45\text{-}55}$, is a gene editing therapy for Duchenne muscular dystrophy. This utilizes AAV delivery of CRISPR/Cas9 to delete *DMD* exons 45-55, thereby restoring the reading frame and restoring dystrophin protein expression. This deletion is associated with one of the most mild Becker mutations seen in patients. Additionally, it covers a hotspot of patient mutations, meaning it would be applicable for around half of all Duchenne patients with a single therapy.

Management Team

Co-founders:
Courtney Young, PhD; Melissa Spencer, PhD; April Pyle, PhD

Board and Other Advisors

SAB members: April Pyle, PhD; Melissa Spencer, PhD; Barry Byrne, MD, PhD; Jeffrey Chamberlain, PhD; Donald Kohn, MD

Presenter Name

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Co-founder and CEO

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Timestamp: Day 2 2:34:04 – 2:53:00

COMPANY PROFILE

Company

Myosana Therapeutics

Indication(s)

DMD

Stage

Preclinical

Pipeline

Duchenne Muscular Dystrophy

Therapeutic Platform

A non-viral platform that targets skeletal and cardiac muscle for delivery of genes of any size.

Management Team

Stanley C. Froehner, Co-Founder

Nicholas P. Whitehead, Co-Founder and Chief Scientific Officer

Steve Runnels, CEO

Board and Other Advisors

Stanley C. Froehner, Chairman of the Board

Nicholas P. Whitehead

Steve Runnels

Presenter Name

Stanley C. Froehner
Co-founder and Chairman of the Board

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View Presentation and Recording
Timestamp: Day 2 5:08:25 – 5:28:17

COMPANY PROFILE

Company

Origent Data Sciences, Inc.

Indication(s)

ALS, Huntington's, Parkinson's, Alzheimer's

Stage

Clinical

Pipeline

We use machine-learning to analyze trials that did not meet their endpoints to identify patient subgroups that could form the basis for a subsequent successful trial. We are currently searching large drug and trial databases (Citeline, ClinicalTrials.gov) for drug candidates that have failed late-stage clinical testing and appear to have been abandoned. We have a list of over 100 drug candidates and are currently in active discussions to examine the clinical trial data sets of half a dozen with the goal of in-licensing promising drugs. Our objective is to rapidly return the drug candidates to phase 2b or phase 3 trials so that they can be registered within 5 to 7 years.

Therapeutic Platform

Our platform is a machine-learning platform. We have developed a number of models that predict disease endpoints commonly used in clinical trials for our target indications. We use these models to develop applications, including virtual controls, prognostic matching, enrichment, randomization, covariate adjustment and subgroup analysis to improve clinical trial efficiency. The key to our platform is our novel, patent-pending method of subgroup analysis that we term "Detectable Effect Cluster" (DEC) analysis. We rank order all patients by predicted outcome, then, starting with small subgroups, systematically expand the patients included in each subgroup using the next most similar patients until all patients are included. The result is a series of overlapping subgroups of increasing size defined by thresholds based on predicted outcomes. The method is computationally efficient and rapidly derived without investigator bias and ultimately includes all the nearest neighbor analyses possible for a given trial. Since the method groups patients that are most similar, it stands a good chance of generating subgroups with significantly less noise than the full analysis set, giving the therapy a better chance to demonstrate an effect size that will yield a significant p-value. We use this innovative approach to methodically identify patient subgroups within a failed clinical trial that could form the basis for a successful subsequent trial.

Management Team

Dave Ennist, PhD, MBA, CEO & Chief Science Officer
Danielle Beaulieu, MPH, Chief Data Science Officer
Albert Taylor, PhD, Director of Research

Board and Other Advisors

Fabian Rosado, MBA, Board Chair
Mike Keymer, MBA, Board Director
Syeed Mansur, Board Director

We have a number of collaborators and paid consultants in ALS, HD, PD and AD diseases as well as in the field of statistics. We are well-known among key opinion leaders in ALS and are building our reputation in HD, PD and AD.

Presenter Name

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COMPANY PROFILE

MDA 2021 CLINICAL & SCIENTIFIC CONFERENCE
Insights in Research Investor Summit

Company

Ortholevo, Inc.

Indication(s)

DMD, SMA, Arthrogryposis, ALS, Stroker, Cerebral Palsy, MS

Stage

Preclinical

Pipeline

Pediatric patients with degenerative neuromuscular disorders, such as muscular dystrophies and other neuromotor diseases, experience muscle tone loss and fibrosis of the joints (i.e. joint contracture). This makes the joints difficult or impossible to bend. This fibrosis, and subsequent loss of mobility, is a recurrent and progressively worsening condition. While there are recently approved treatments addressing the primary neuromuscular disease, there are no effective treatments for the frozen joints that inevitably accompany nearly all neuromotor diseases. Our therapeutic boasts a platform opportunity with broad clinical utility: fibrosis is common to many diseases, thus our follow-on markets are much larger than SMA, DMD, and CP alone. Our pipeline fibrotic indications based around the same therapeutic platform include 1) musculoskeletal fibrotic conditions (frozen shoulder, Dupuytren's contracture, Peyronie's disease, and plantar fibromatosis), 2) pulmonary complications from COVID-19 (lung fibrosis), and 3) aesthetic fibrotic conditions (cellulite and scars).

Therapeutic Platform

Our solution for patients and their families is a quick, safe, office-based injection into contracted joints, allowing them to move freely. Our single-injection therapeutic eliminates joint stiffness for a prolonged duration due to its local sustained-release technology. The sustained-release depot formulation's antifibrotic agent is the peptide relaxin, a human hormone. Relaxin has native antifibrotic properties to maintain connective tissue homeostasis; by delivering an effective local relaxin concentration to the fibrotic tissues of contracted joints, our therapeutic achieves a sustained potent response without systemic side effects. By achieving sustained relaxin receptor activation, our product triggers a controlled breakdown of fibrous adhesions, restoring joint range of motion. This phenomenon is similar to how relaxin (upregulated during pregnancy) naturally functions to prepare the body for childbirth, by loosening fibrous connective tissue of the pelvic ligaments. This peptide therapeutic formulation provides an unprecedented opportunity to treat joint contracture, which no other drug or non-drug treatment can provide. Our product will be the first treatment for stiff joints based on reversing the underlying fibrous tissue pathology.

Management Team

Edward Ahn, Ph.D. - CEO
Colin White, Ph.D. - CSO
Davey Bakhshi - Corporate Development Officer
Benjamin Cooper, Ph.D. - R&D Manager

Board and Other Advisors

Mark Grinstaff, PhD
Ara Nazarian PhD
Basil Darras, MD
Brian Snyder, MD, PhD
Edward Rodriguez, MD, PhD
Ross Bathgate, PhD

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CEO

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View Presentation and Recording
Timestamp: Day 2 5:28:20 – 5:42:20

COMPANY PROFILE

Company

PathMaker Neurosystems Inc.

Indication(s)

ALS

Stage

Preclinical

Pipeline

PathMaker Neurosystems is developing a pipeline of first-in-class neuromodulation products for serious neurological disorders. PathMaker's first product, MyoRegulator®, is the world's first non-invasive neuromodulation device for the treatment of spasticity. It is the first and only neuromodulation device treating muscle spasticity without the need for drugs or surgery. It was one of the first devices to be designated by the U.S. FDA as a "breakthrough" medical device and has completed clinical studies in the U.S. and in Europe. PathMaker is leveraging the MyoRegulator® technology to develop a novel approach to ALS that can provide not only symptomatic therapy, but potential disease-modifying activity. We have developed a robust pre-clinical data package in the SOD1-G93A mouse model of ALS supporting functional improvement as well as increased survival following treatment.

Therapeutic Platform

Our company's multi-site DCS (direct current stimulation) platform non-invasively stimulates at multiple sites along the neural axis to induce simultaneous current flow across the spinal cord and down the afflicted limb, resulting in suppression of hyperexcitable spinal motor neurons. We recently reported the first direct link between overexpression of a specific neuronal co-transporter, NKCC1, and the emergence of spasticity, a condition associated with motor neuron hyperexcitability (Mekhael et al., 2019). NKCC1 is a Na-K-Cl cotransporter found on motor neurons and is involved in maintaining chloride gradient. We reported that in an animal model of spinal injury, NKCC1 becomes elevated after injury resulting in neuronal hyperexcitability and increased spasticity. We reported that our multi-site DCS technology suppressed NKCC1 levels, reduced excitability and reduced spasticity. We have recently found that NKCC1 is elevated in SOD1G93A mice, and that stimulation with our technology results in reduction of NKCC1 levels, improvement in motor function, and increased survival (unpublished). We have further found direct effects on biochemical markers associated with ALS after stimulation, including SOD1 and HSP70.

Management Team

Nader Yaghoubi, M.D., Ph.D. is President, Chief Executive Officer and Co-Founder

Jerry Jennings, B.S.E.E. is Chief Technology Officer

Sheila Hemeon-Heyer, J.D. is Vice President of Regulatory and Clinical Affairs

Board and Other Advisors

BOARD OF DIRECTORS
Hooman Hakami, B.B.A. is Chairman of the Board
Terry Bresenham, M.S. is a Board Director
Jake Maslow, J.D. is a Board Director

SCIENTIFIC ADVISORY BOARD
Zaghloul Ahmed, Ph.D. – CUNY/CSI (scientific founder)
Jean-Charles Lamy, Ph.D. – Paris Brain Institute (ICM)
Emilio Bizzi, M.D., Ph.D. – MIT
Ole Isacson, M.D.-Ph.D. – Harvard
Bechir Jarraya, M.D., Ph.D. – Foch Hospital, CEA Neurospin

Presenter Name

Nader Yaghoubi, M.D., Ph.D.
President and CEO

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Timestamp: Day 1 4:06:43 – 4:24:10

COMPANY PROFILE

Company

Prosetta Biosciences, Inc.

Indication(s)

fALS and sALS

Stage

Preclinical

Pipeline

Assembly modulators for multiple therapeutic areas all in preclinical development

Therapeutic Platform

Catalyzed assembly modulation

Management Team

Vishwanath R. Lingappa, CEO and CTO
Suganya Selvarajah Director of Neuroscience

Board and Other Advisors

Dale Bredesen
Jeffrey Rosenfeld

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Timestamp: Day 1 4:48:24 – 5:08:25

COMPANY PROFILE

Company

Raya Therapeutic, Inc.

Indication(s)

ALS

Stage

Preclinical

Pipeline

Raya Therapeutic is putting together a portfolio of 4 to 5 compounds, with ALS as the primary indication, and planning to develop them in a single P3 trial (in a platform umbrella design) in 2022. All are clinical stage, small molecules and have different MOAs.

Therapeutic Platform

One of the unique aspects of this company is the ability and objective to test combinations of the different compounds to look for synergistic effects. The company has already started testing each compound in vitro on their own and will eventually test them in combination. The most optimal combinations will be tested in a P1B trial and if warranted in P3.

ALS is a heterogenous disease and it is likely going to take a combination of different drugs to help turn this disease from a deadly one into a chronic one.

Management Team

Anjan Aralihalli, Founder
Kim Staats, Scientific Advisor

Board and Other Advisors

Dr. Angela Genge (Montreal Neurological Institute)

Presenter Name

Anjan Aralihalli
Founder

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COMPANY PROFILE

MDA CLINICAL & SCIENTIFIC CONFERENCE 2021
Insights in Research Investor Summit

Company

Sea Pharmaceuticals, LLC

Indication(s)

sALS

Stage

Preclinical

Pipeline

Sea Pharma has created and is preclinically-characterizing proprietary synthetic small molecule selective AMPAR ion channel antagonist motor neuron protectants as potential treatments for sporadic ALS. Sea is on the cusp of advancing its first lead molecule into clinical development for sALS (i.) SPM-0404 oral. Sea's second compound (ii.) SPM-0303 parenteral is close behind (both are late stage nonclinical). Sea's third and fourth molecules (iii.) another oral and (iv.) another parenteral (both are mid-stage nonclinical backups). All 4 of these molecules are Sea Pharma proprietary intellectual property, and they are protected by chemical composition of matter and therapeutic use patent applications filed at USPTO in 2020 and in 2021.

Therapeutic Platform

Sea Pharma created a proprietary platform of chiral chemistry and has synthesized several proprietary molecules: selective AMPAR ion channel antagonists for the treatment of the neurological disease Sporadic ALS (sALS) and the treatment of chronic tinnitus. All nonclinical work by Sea is focused on sALS. Sea's molecules are tested for (A.) biological activity and selectivity, respectively, in vitro in slice whole cell-single neuron electrophysiology recording assays, (B) efficacy in vivo in rodent models of seizures and sALS, and (C) suitable pharmaceutic and pharmacological properties. New Ph.2 clinical trial (literature data) demonstrated AMPAR antagonist clinical proof of concept in chronic tinnitus patients.

Management Team

James P. Pearson Ph.D. CEO, Chief Scientific Officer, President, biologist, pharmacologist, investor, founder

Eduardo J. Martinez Ph.D. VP Sea Pharma LLC, medicinal chemist, process chemist, co-founder

Board and Other Advisors

Board
J.P. Pearson Ph.D.
Ben F. Cravatt Ph.D.
Eric R. Olson Ph.D.
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Nabil Elkouh, Ph.D.
Michael J. Higgins M.B.A.
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W. Kendall Brown J.D.

KOL - Clinical
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Paul Grint M.D.

KOL - Neuroscience
Jim E. Huettner Ph.D.
Michael P. Kavanaugh Ph.D.
Shin Kwak M.D., Ph.D .
Tony Yaksh Ph.D.
Aron H. Lichtman Ph.D.
H. Steve White Ph.D.

KOL - Chemistry
Paul L. Ornstein Ph.D.
Chris H. Senanayake Ph.D.
Joseph D. Armstrong III Ph.D.

Pharmaceutical Advisors
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Eric R. Olson Ph.D.
Chris H. Senanayake Ph.D.
John E. Sagartz D.V.M., Ph.D.

Presenter Name

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Timestamp: Day 1 3:43:00 – 4:06:40

MDA CLINICAL & SCIENTIFIC CONFERENCE 2021
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COMPANY PROFILE

Company

Sola Biosciences

SOLA BIOSCIENCES

Indication(s)

ALS

Stage

Preclinical

Therapeutic Platform

Sola Biosciences has developed a novel technology termed "JUMP70" that can specifically control cellular protein folding and quality control systems for targeted proteins. JUMP70 technology is based on the structure of Engineered J-domain protein that carries J-domain sequence for recruitment and activation of Hsp70 chaperone system and target protein binding sequence for chaperone activation specifically to the targeted disease-causing protein. We have shown that Engineered Chaperone technology can strikingly manipulate the protein folding process to specifically target several misfolded proteins such as mutant huntingtin and alpha-synuclein, and TDP-43 proteins.

Pipeline

Sola Biosciences is a biotherapeutics company focusing on developing transformative therapies to treat protein misfolding diseases. Sola's invention, JUMP70 technology, is designed to harness the power of a patient's own chaperones to repair misfolded proteins that cause devastating neurodegeneration in diseases such as ALS, Huntington disease and Parkinson's disease.

Preliminary data in cultured cells show that Engineered J-domain protein designed for pathogenic TDP-43 (hereinafter referred to as SOL-257) strikingly accelerated protein degradation process of pathogenic TDP-43 proteins without affecting normal TDP-43, leading to our belief that JUMP70 technology to target pathogenic TDP-43 can be developed as a promising treatment for ALS patients. In this study, we propose in vivo efficacy study of SOL-257 to assess the effect on neurodegeneration, behavioral abnormalities, survival, and TDP-43 pathology using TDP-43 model mice.

Management Team

Keizo Koya, Ph.D. (Founder and CEO)
Akinori Hishiya, Ph.D. (Founder and CSO)
Gerald F. Cox, M.D., Ph.D. (Acting Chief Medical Officer)
Eugene Kim, J.D., Ph.D. (Head, IP and Corporate Strategy)
Donald P. Andrade (Chief Financial Officer)

Board and Other Advisors

Gerald F. Cox, M.D., Ph.D.
Clotilde Lagier Tourenne , M.D., Ph.D.
Miguel Sena-Esteves, Ph.D.

Presenter Name

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Timestamp: Day 1 4:24:15 – 4:48:15

MDA CLINICAL & SCIENTIFIC CONFERENCE 2021
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COMPANY PROFILE

Company

Thera Neuropharma Inc.

Indication(s)

ALS, Traumatic brain injury, Alzheimer's, other

Stage

Preclinical

Pipeline

Thera Neuropharma is developing a new class of small molecules (SMRT) and a self-delivering RNA interference compound library targeting the SOD1 protein (SOD1-RNAi) for treatment of neurodegenerative diseases (NDD), initially focusing on Amyotrophic Lateral Sclerosis (ALS) and later expanding to traumatic brain injury (TBI) and eventually Alzheimer's Disease (AD) or other NDD. Our objective is to leverage the complementary characteristics of both technologies and develop them as disease-modifying therapeutics. Our SMRT leads are positioned to enter IND-enabling program and the SOD1-RNAi is at lead selection stage. We intend to move both technologies separately through IND and advance, by 2022, our SMRT lead candidate to a clinical study in ALS patients leveraging the opportunity to test a predictive biomarker.

Therapeutic Platform

Two platforms: Small molecule regenerative technology [SMRT]; self-delivering RNA interference® technology [sd-RNAi]. Thera is developing the first and only small molecule regenerative technology (SMRT) leveraging the therapeutic potential of a key brain factor, nuclear factor (NF)-κB p65, and a critical mitochondrial enzyme, manganese-superoxide dismutase (MnSOD). SMRT technology is associated with strong neurotrophic and neuroprotective effects and exert disease modification effects by protecting neurons against cell toxicity, inducing brain network regeneration, and maintaining functionality by delaying the progression of symptoms of ALS and TBI. Therapeutic properties similar to our compounds have not been shown in any marketed product or experimental compound for ALS or TBI. SOD1 self-delivering hsiRNAi combines features of RNAi and antisense technologies. Our hsiRNAi oligonucleotides target a key ALS pathogenic gene (SOD1) significantly decreasing SOD1 protein expression and potentially exerting a therapeutic effect in both genetic and non-genetic forms of ALS.

Management Team

Antonella Favit-VanPelt, M.D., Ph.D., Founder, President & CEO
Guy Maestre, Pharm.D, Founder, Chief Operating Officer
William Vincek, Ph.D., Sr. Vice-President of CMC & QA Manufacturing
Zdzislaw Wieckowski, General Counsel

Board and Other Advisors

Antonella Favit-VanPelt, M.D., Ph.D., Chairwoman of the Board
Guy Mestre, Director
Geert Cauwenberg, MBA, Director
Fran Lessans, RN, Owner, President and CEO, Passport Health, Observer

Presenter Name

Antonella Favit-VanPelt, MD, PhD
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Timestamp: Day 2 4:40:27 – 4:57:50

COMPANY PROFILE

Company

Toleranzia AB

Indication(s)

Myasthenia gravis

Stage

Preclinical

Pipeline

Myasthenia gravis

ANCA vasculitis

Therapeutic Platform

Recombinant protein based antigen specific immunotherapy

Management Team

Charlotte Fribert, Chief Executive Officer, CEO
Björn Löwenadler, Chief Business Officer, CBO
Vidar Wendel-Hansen, Chief Medical Officer, CMO
David Wahlund, Chief Financial Officer, CFO

Board and Other Advisors

Anders Milton - Chairman of the Board
Maarten Kraan
Eva Lindgren
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MDA CLINICAL & SCIENTIFIC CONFERENCE 2021
APRIL 29, 2021
Insights in Research Investor Summit

COMPANY PROFILE

Company

Treventis

TREVENTIS™ CORPORATION

Indication(s)

ALS

Stage

Preclinical

Pipeline

Our lead program [candidate declaration] focuses on anti-misfolding small molecules in tauopathy (3R, 4R, familial mutations and mixed) with relevance to Alzheimer's disease. We have further CCM-driven efforts in ALS [lead optimization] (small molecule inhibitors of misfolded TDP isoforms – sporadic ALS and FTD focus) and in oncology [hit-to-lead] (small molecule inhibitors of misfolded p53 – metastatic cancer focus) and other neurodegenerative diseases that show the wide utility of our technology platform.

Therapeutic Platform

TREVENTIS™ Corporation is dedicated to treating and preventing protein misfolding diseases. We utilize a proprietary, patent-pending discovery engine – Common Conformational Morphology (CCM) – to identify druggable active sites in misfolded protein targets. CCM combines unique in silico models with deep expertise in model development (in vitro, ex vivo, in vivo) to enable rational drug design against misfolded protein targets. With its groundbreaking small molecule anti-misfolding therapeutics programs, Treventis aims for a world with disease-modifying treatments for all protein misfolding diseases.

Management Team

CHRISTOPHER J. BARDEN
Chief Executive Officer

DONALD F. WEAVER
Chief Medical Officer

MARK REED
Chief Scientific Officer

MARCIA TAYLOR
VP Research

CARLOS ZEPEDA
Chemistry Group Leader

SEUNG-PIL YANG
Biology Group Leader

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Independent Director
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Independent Director
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Independent Director
WILLIAM WONG, PH.D.
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