MARCH 13, 2022

2nd Annual MDA Insights in Research Investor Summit

2022 MDA CLINICAL & SCIENTIFIC CONFERENCE

Insights in Research Investor Summit

*(*presenting companies are in **BOLD**)*

MDA CLINICAL & SCIENTIFIC CONFERENCE 2022
Insights in Research Investor Summit

2nd Annual MDA Insights in Research Investor Summit (IRIS) for Neuromuscular Disease

All times listed are Central Daylight Time

Room Hermitage CD

Sunday, March 13, 2022	
9:00 AM – 9:10 AM	Opening Remarks
Muscular Dystrophy Focused Opportunities	
9:10 AM – 9:30 AM	***Regeneration from Within*** Ryan Mitchell, PhD Satellos Bioscience Inc.
9:30 AM – 9:50 AM	***MyoGene Bio's Gene Editing Therapy for Duchenne*** Courtney Young, PhD MyoGene Bio
9:50 AM – 10:10 AM	***MyoArete's Utrophin-Upregulation Small Molecules for Muscular Dystrophy*** Tejvir S. Khurana, MD, PhD MyoArete
10:10 AM – 10:30 AM	***A Novel Platform for Non-Viral Targeted Delivery of Full-Length Dystrophin to Dystrophic Muscle*** Stanley C. Froehner, PhD Myosana Therapeutics, Inc
Break	
10:30 AM – 11:00 AM	
11:00 AM – 11:20 AM	***Anti-fibrotic joint injection to restore range of motion to patients with arthrofibrosis (joint contracture)*** Edward Ahn, PhD Ortholevo
11:20 AM – 11:40 AM	***Innovating Therapies for Neuromuscular and Heart Diseases*** Casey Childers, PhD. Kinea Bio
Other NMD Focused Opportunities	
11:40 AM - 12:00 PM	***Novel Small Molecules Rescue 3 mouse models of mitochondrial disease: Leigh Syndrome, Friedreich's, and LHON*** Gino Cortopassi, PhD Myto Therapeutics
12:00 PM - 12:20 PM	***Novel Small Molecule Calcium Channel Gating Modifiers with Broad Therapeutic Potential at the NMJ*** Stephen Meriney, PhD University of Pittsburgh
Lunch on Your Own	

12:20 PM - 1:30 PM	
ALS Focused Opportunities	
1:30 PM - 1:50 PM	***Clene Nanomedicine, a Novel Approach to Improving Neurological Function*** Karen Ho, PhD Clene Nanomedicine, Inc
1:50 PM - 2:10 PM	***Patient-Based Drug Discovery for Neurodegenerative Diseases*** Sam Alworth, PhD AcuraStem
2:10 PM - 2:30 PM	***Developing HDAC6 Inhibitors as Novel Therapeutics for ALS*** Janice Kranz, PhD Eikonizo Therapeutics, Inc
2:30 PM - 2:50 PM	***Advanced predictive analytics for clinical trials*** David Ennist, PhD Origent Data Sciences
2:50 PM – 3:10 PM	***Towards personalized medicine: DMD disease modeling with 3D muscle tissues*** Nicholas Geisse, PhD Curi Bio

MDA® Muscular Dystrophy Association

MDA VENTURE PHILANTHROPY

MDA RESEARCH DEPARTMENT

INDUSTRY FUNDING OPPORTUNITIES

MDA Venture Philanthropy Program (MVP)

The MVP program targets investments supporting drug development for neuromuscular disease in promising early-stage neuromuscular research, serving to de-risk development and help enable a Series A funding.

MDA's MVP Program has funded: Aavanti, Myogene, Locana, AcuraStem, Iron Horse (nVector).

FOR INDUSTRY FUNDING INQUIRIES, PLEASE EMAIL BCRISWELL@MDAUSA.ORG.

MDA Research Team:
Sharon Hesterlee, PhD - Chief Research Officer
Angela Lek, PhD - VP of Research
Edritz Javelosa, PhD - Director, Research Portfolio
Evrim Atas, PhD - Director, Research Portfolio
Elizabeth Habeeb-Louks, MSc - Manager, Grants
Bryan Criswell - Manager, Grants

Company Profile

AcuraStem
PATIENT-BASED DRUG DISCOVERY

AcuraStem

Stage
IND-Enabling Studies

Indications

ALS, Charcot-Marie-Tooth (CMT) disease, frontotemporal dementia (FTD)

Therapeutic Platform

AcuraStem is pioneering patient-based drug discovery and ultimately how treatments are developed for neurodegenerative diseases—including sporadic ALS—using its proprietary, best-in-class, disease-modeling platform, iNeuroRx®. The iNeuroRx® technology platform combines patient-derived disease models with human genetic data. It provides target discovery and efficacy profiling in patient-derived models using machine-learning enabled assays. All discovery and validation is done with human models and data. The platform has been validated through extensive collaborations and successful translation to animal models. Currently the platform comprises over 80 patient-derived models and assays covering ALS, FTD and CMT diseases. In addition, AcuraStem has developed a proprietary antisense oligonucleotide (ASO) design and development technology that enables in silico screening and ranking of candidates based on their likely manufacturability and safety.

Pipeline

AS-1 Program (PIKFYVE): AcuraStem discovered PIKFYVE inhibition as a novel mechanism to clear misfolded protein aggregates and prevent neurodegeneration in ALS patient models. It could be the first disease-modifying therapy for sporadic ALS / FTD, an extremely high unmet need population. A first-in-class antisense oligonucleotide (ASO) development candidate, AS-202, is currently in IND-enabling studies. It also has encouraging data in CMT, FTD and other neurodegenerative indications.

AS-2 Program (SYF2): ASO-mediated suppression of SYF2 improves RNA splicing in the context of TDP-43 pathology to enhance normal TDP-43 function and reduce neurotoxic TDP-43 aggregates. Currently in lead optimization stage.

AS-3 Program (confidential target): AS-3 is a first-in-class ASO that addresses one of the most significant genetic risk factors in ALS / FTD. The iNeuroRx® technology provides a competitive advantage for the optimization of ASOs that are efficacious in patient neurons for this hard-to-target gene.

IP Status

AcuraStem has eleven patent applications and one approved US patent covering its platform, therapeutic targets and compositions of matter.

Goals for Presentation

AcuraStem seeks to meet with investors, funding organizations and collaborators that can help with its mission to bring these transformative treatments to patients.

Presenter Name

Sam Alworth, MS, MBA
Co-Founder and CEO

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Company Profile

Aquilus Pharmaceuticals

Aquilas Pharmaceuticals

Stage
Grants/Stipends, Angel

Indication
ALS

Therapeutic Platform

The company has a portfolio of very potent & proprietary matrix metalloproteinase (MMP) inhibitors. This includes its lead dual acting MMP-2/-9 inhibitor (AQU-118) for the treatment of ALS and neuropathic pain. AQU-118 is in late-stage, preclinical development and will enter clinical development for the treatment of ALS in first quarter 2023.

Pipeline

Aquilus Pharmaceuticals (Aquilus) is a biotech company focused on the treatment and management of ALS, neuropathic pain and other neurological disorders. The company has a portfolio of very potent & proprietary matrix metalloproteinase (MMP) inhibitors. This includes a dual acting MMP-2/-9 inhibitor program (AQU-118) for the treatment of Amyotrophic Lateral Sclerosis (ALS) and neuropathic.Aquilus' dual active MMP-2/-9 inhibitor affects various key inflammatory pathways that reduce nerve injury and cell death (apoptosis). It has exhibited very good safety in mice, rats and dogs. Aquilus' primary strategy is to pursue clinical development of its lead MMP-2/-9 inhibitor, AQU-118, as a first in class treatment for ALS. Aquilus plans to complete investigational new drug (IND) enabling studies for AQU-118 by the end of 2022 and submit its completed IND to the FDA by the first quarter of 2023. Aquilus is looking for investors to help fund early clinical development of AQU-118 for the treatment of ALS.

IP Status

Aquilus Pharmaceuticals has fully issued composition of matter IP around AQU-118 in the US, Canada, Europe, China, Japan, Russia, Korea, Israel and Australia. It has filed new method of use IP for AQU-118 for the treatment of neurodegenerative disorders.

Goals

Aquilus is currently completing its IND enabling studies for AQU-118 for the treatment of ALS. It currently has sufficient grant funding through a grant from the ALS Association and a government grant to complete all of the remaining IND enabling studies. It hopes to file its IND application in the first quarter of 2023 and upon FDA approval of the IND begin clinical testing of AQU-118 in people with ALS (PALS). It is looking for either a series A round or a biotech partner to help fund the proof of concept clinical trial of AQU-118 in people with ALS.

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Company Profile

Clene Nanomedicine, Inc

Stage
NASDAQ: CLNN

Indications

ALS, Parkinson's Disease, and Multiple Sclerosis

Therapeutic Platform

Clene Nanomedicine is a clinical-stage biopharmaceutical company focused on revolutionizing the treatment of neurodegenerative diseases with a proprietary potential first-in-class nanotherapeutics platform. Clene's approach to address the CNS energetic needs in disease begins with platform technology integrating physics with biology, which has allowed us to explore broad applications of our drugs across therapeutic areas.

Pipeline

Clene is advancing a pipeline of nanotherapeutics to address high unmet medical needs in Amyotrophic lateral sclerosis (ALS), Multiple Sclerosis (MS) and PD (Parkinson's disease). Our lead asset, CNM-Au8®, is an oral suspension of clean-surfaced, catalytically-active gold nanocrystals developed to restore neuronal health and function by increasing energy production and utilization.

CNM-Au8 is currently being investigated as a disease-modifying treatment in the first-of-it-kind phase 2/3 HEALEY ALS platform trial in ALS led by The Healey Center for ALS at Mass General Hospital with results expected the second half of 2022 and in stable relapsing MS patients in a phase 2 trial, VISIONARY-MS, with results expected second half of 2022. Plans to initiate a Phase 2 trial in PD is planned for second half of 2022.

Strong Intellectual Property

Based on our unique approach to drug development, Clene has secured over 150 patents issued worldwide for states of matter, methods of manufacturing, devices for manufacturing and methods of treatment.

Goals for Presentation

Increase awareness and investor knowledge of Clene's progress in neurodegenerative disease drug development and invite discussion for potential partnership opportunities.

Presenter Name

Karen Ho, PhD
Head, Translational Medicine

Jennifer Hotchkin
Head, Commercial

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Company Profile

Curi Bio

Indication

DMD, MD1, other related muscular disorders - cardiac and muscle

Stage

Grants/Stipends, Venture Capital Series A

Therapeutic Platform

Curi Bio is integrating human iPSC-derived cells, tissue-specific biosystems, and AI-enabled data analytics to accelerate the discovery of new therapeutics. We support preclinical candidate screening in human primary and stem cell-derived cardiac and skeletal muscle tissue (disease modeling). Our skeletal muscle system allows direct measurement of contractile force (twitch and tetanic force, muscle fatigue). DMD and DM1 engineered tissues show significant deficits in muscle function compared to healthy isogenic controls.

Pipeline

Focus on DMD, DM1, XL-MTM, Nemaline myopathies, etc.

IP Status

We have numerous patents and/or trade secret on our skeletal muscle model, various instrumentation and data analysis of the muscle constructs, etc.

Goals for Presentation

Present Curi Bio as a scientific partner to accelerate late stage preclinical research and provide more clinically-relevant decision making using the "Curi Engine". - "Match making" with investors possibly looking to pair companies with other preclinical technologies or clinical therapeutic modalities and/or delivery technologies in the gene therapy space. - Partner with investors and patient advocacy groups in the muscular disorder space to help provide additional disease (tissue) models to the industry.

Presenter Name

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CSO

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Company Profile

Eikonizo Therapeutics

Indication
ALS

Stage
Series A Funding

Description of Therapeutic
Eikonizo is a biotech company with unique expertise in designing novel, brain-penetrant HDAC6 inhibitors as therapeutics for neurodegenerative diseases. The name 'histone deacetylase 6' is a misnomer, as HDAC6 does not deacetylate histones. Instead, HDAC6 is a cytoplasmic enzyme that deacetylates tubulin and other proteins, including several involved in ALS pathology. Inhibiting HDAC6 is a novel approach to improve microtubule resilience and restore deficient axonal transport in neurons of ALS – thus improving multiple pathways and ultimately slowing or stopping disease progression. Our first program on selective HDAC6 inhibitors focuses on ALS, but emerging biology suggests HDAC6 inhibition can have therapeutic benefits in other neurodegenerative diseases (Alzheimer's disease, chronic traumatic encephalopathy and chemotherapy-induced cognitive impairment).

Description of Platform
A core tenet of Eikonizo's drug discovery/development path features establishing target engagement early by using proprietary, specific companion PET imaging agents. Our HDAC6 program includes an HDAC6 PET tracer that we have validated in a Phase 1 clinical trial. HDAC6 PET imaging supports direct visualization and quantitation of target engagement in the brain of living patients, allowing for precise dose optimization. This strategy thus enables us to advance assets faster and more cost-effectively with less risk. Our vision is to develop HDAC6 inhibitor assets for multiple indications, both CNS and non-CNS, all of which can benefit from using the companion HDAC6 PET tracer to both quantify target occupancy and optimize human dose selection. Once we advance HDAC6 programs to the clinic, we will apply the same successful approach to additional post-translational modifying enzymes.

IP Status
Strong IP position encompassing composition of matter (COM) and methods of use for two distinct scaffolds and for both brain-penetrant and peripherally-restricted compounds. Specifically: (1) A granted US COM patent, protection through 2037; (2) An exclusive license from MGH for COM including the PET agent, protection through 2038; and (3) Fully owned COM global filings through 2040 and 2042; (4)Two additional provisional applications to be filed by Q2 2022.

Goals for Presentation
I am primarily interested in learning if and how our story resonates with the MDA IRIS audience. One of our goals is to field as many tough questions as possible. We hope our expanded data package will spark great conversation around the therapeutic hypothesis, but we also want to understand the perceived gaps in our data package, or what needs to be more clearly communicated. An additional overarching goal is to engage MDA members to continue the conversation throughout the conference and, ideally, afterwards as well.

Presenter Name
Janice Kranz, PhD
Co-Founder and CSO

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Company Profile

KINEABIO

Kinea Bio

Stage
Friends and Family

Indications

LGMD type 2i/R9, heart failure, DMD, myotonic dystrophy type 1

Therapeutic Platform

The scientific founders at Kinea Bio have extensive background in AAV-based gene therapy, neuromuscular disorders, and related fields. Kinea Bio will combine proprietary muscle-specific promoters, myotropic capsids, and optimized genetic cargoes to produce industry-leading gene therapies. Additionally, through a joint venture with Curi Bio, Kinea Bio has access to numerous disease models and cell lines to customize treatments, study disease severity, and develop potency assays.

Pipeline

Kinea currently has four indications with in vivo data. Selection of a development candidate and IND-enabling studies will be taking place in 2022 for our lead program. We are performing preclinical studies for our second program this year and expect to have a developmental candidate by early 2023. Our third program is well established, and we expect to select a development candidate by late 2023. Licensing is being finalized for our fourth program, and we expect to begin preclinical studies late this year. Numerous additional indications are also being considered.

IP Status

Kinea Bio has secured exclusive license and non-exclusive license to platform and pipeline assets, including granted and pending patent portfolios for related technologies.

Goals for Presentation

We would like to introduce Kinea Bio to the NMD community. Many of our founders have been involved in the NMD community for decades, both in a research and in a clinical sense. However, by combining their expertise, they have the opportunity to produce a gene therapy for multiple muscular dystrophies and other indications. Our presentation will highlight the strengths of our platform and give us an opportunity to answer any questions that may arise. Ultimately, we would like to attract investors and inform the attendees about Kinea Bio.

Presenter Name

Casey Childers, DO, PhD
CEO

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Company Profile

Mito Therapeutics

Indications

Friedreich's ataxia, Leigh Syndrome

Stage

Grants/stipends, Angel

Therapeutic Platform

small molecule

Pipeline

MYT-109 for Friedreich's ataxia (FA) , Leigh Syndrome (LS), Leber's hereditary Optic Neuropathy (LHON).

IP Status

Composition of matter patent issued July 2021, 19.4 years of patent life, extensions through use indications and Orphan disease filings possible.

Goals for Presentation

To introduce Investors to a new Mitochondrial-focused therapeutic, that increases function and rescues lethality in 3 mouse models of 3 different mitochondrial diseases, Leigh Syndrome, Friedreich's ataxia, and Leber's hereditary Optic Neuropathy

Presenter Name

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Company Profile

MyoArete

Stage

Indications

Duchenne Muscular Dystrophy

Therapeutic Platform

Small Molecule
RNA
DNA

Pipeline

MyoAr Small Molecules
MyoAr SBOs
MyoAr Gene Edit

IP Status

IP filed. All owned and managed by Univ. of Pennsylvania

Goals for Presentation

To present to potential investors

Presenter Name

Tejvir S. Khurana, MD, PhD.
Founder & CEO

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Company Profile

MyoGene Bio

MyoGene Bio

Stage
Grants/Stipends

Indications
Duchenne Muscular Dystrophy

Therapeutic Platform

MyoDys45-55 is a gene editing therapy that uses AAV-mediated delivery of CRISPR/Cas9 to permanently remove exons 45-55 in the DMD gene, thus restoring the reading frame for ~50% of Duchenne patients and generating a deletion that has been associated with one of the mildest versions of Becker muscular dystrophy. We are testing this in combination with immune suppression to improve safety and efficacy aspects with the potential for redosing.

Pipeline

Initial approach, MyoDys45-55, for Duchenne muscular dystrophy with redosing
Potential applications for other muscle diseases including LGMD, FSHD, myotonic dystrophy

IP Status

MyoGene has an exclusive license for 2 filed PCT patent application for use in Duchenne and Becker. One is on the 45-55 gene editing approach with claims to cover specific guide RNA sequences as well as the idea of a large exon 45-55 deletion (greater than 330kb). The second is for other gene editing strategies in the DMD gene for smaller subsets of patients.

Goals for Presentation

Networking and current seed funding raise to supplement our awarded $3.4M CIRM grant to advance preclinical development of MyoDys45-55 as well as support additional large animal model studies

Presenter Name

Courtney Young, PhD
Co-founder and CEO

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Company Profile

Myosana Therapeutics

Stage
Pre-seed investments

Indications
Duchenne Muscular Dystrophy

Therapeutic Platform

Myosana has developed a novel non-viral gene delivery platform that targets genes of any size to skeletal and cardiac muscle. The platform overcomes the key limitations associated with AAV gene delivery and allows redosing of the gene as required. The platform is applicable to a wide range of genetic diseases of skeletal and cardiac muscle.

Pipeline

Duchenne Muscular Dystrophy
Undisclosed Additional Indication

IP Status

A patent covering composition of matter and methods of use is pending. Myosana holds an exclusive option to license and is in the process of executing the license from the University of Washington.

Goals for Presentation

- Describe the properties of the Myosana platform that target gene expression in skeletal and cardiac muscle.
- Provide data demonstrating that multiple dosing results in the expression of higher levels of full-length dystrophin
- Present functional studies that demonstrate a marked improvement in diaphragm muscle contractile function in the mdx4v mouse model.
- Identify investors for seed or Series A funding

Presenter Name

Stanley C Froehner, PhD
Co-Founder and Chairman

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Company Profile

ORIGENT DATA SCIENCES

Origent Data Sciences

Stage
Angels, Grants, Clients

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

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, patented 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 rae included. The result is a series of overlapping subgroups of increasing size defined by thresholds based on predicted disease progression. 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.

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 several others 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.

IP Status

Our ALS, Huntington's, Parkinson's and Alzheimer's models are held as trade secrets. The method and the system for how we implement our novel subgroup analysis technology (Detectable Effect Cluster (DEC) analysis was awarded a US patent on Oct 5, 2021 (Systems and Methods for Designing Clinical Trials, US patent #11,139,051) and patents are pending in Europe, Japan and Canada.

Goals for Presentation

Identify investor(s) for our Series A round.

Presenter Name

David Ennist, PhD
CEO and Chief Science Officer

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Company Profile

Ortholevo

Stage
Grants/Stipends

Indications

Arthrofibrosis (joint contracture) resulting from muscular dystrophies

Therapeutic Platform

Relaxin is an agonist of the receptor RXFP1, a G-protein-coupled receptor that plays a role in regulation of fibrosis and extracellular matrix remodeling. When relaxin activates RXFP1, this triggers a two-fold response to restore functional range of motion: it is both antifibrotic (breaks down fibrous adhesions by upregulating matrix metalloproteinases), and antifibrogenic (decreases synthesis of new collagenous fibrous tissue by inhibiting Smad2 phosphorylation and decreasing differentiation of alpha smooth muscle actin).

Pipeline

Ortholevo is developing a pipeline of intraarticular (injected into the affected joints) therapeutics that deliver a sustained effective drug concentration of the antifibrotic peptide relaxin. This signaling peptide, present in both males and females and upregulated during pregnancy to loosen pelvic ligaments and joints, has demonstrated a strong safety profile upon systemic administration in clinical trials. We are developing products for indications in muscular and neuromuscular disorders, pulmonary fibrosis, aesthetic fibrotic conditions, and other musculoskeletal indications.

IP Status

We are executing an exclusive license from Boston University and Beth Israel Deaconess Medical Center for the intellectual property portfolio developed there by Ortholevo's™ academic founders. Two patents (one related to composition of matter and the other on methods of patient treatment) have been granted, with an additional patent pending on product manufacturing methods. These patents also contain broader defensive compositions and treatment strategies to prevent competition related to this technology.

In addition to patent protection, we have developed significant know-how regarding product manufacturing processes and synthetic conditions, which poses a barrier against competing manufacturers; sustained-release microparticle drug formulations require substantial manufacturing process development, and this has historically prevented competition in this technological space..

Goals for Presentation

We currently have early-stage funding from the Department of Defense's medical research program's Duchenne Muscular Dystrophy Research Program, and we are seeking follow-on funding to expedite this product's path to the clinic. By networking with corporate BD parties and investors, we are aiming to advance this product and grow our business by tapping into the resources that the MDA IRIS assembles.

Presenter Name

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CEO

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Company Profile

PATHMAKER NEUROSYSTEMS

PathMaker Neurosystems, Inc

Stage
Grants/stipends, Angel

Indication
ALS

Therapeutic Platform
Recent research has established important links between ALS and motor neuron hyperexcitability. We have developed a novel non-invasive approach that uses multi-site DCS (direct current stimulation) to suppress hyperexcitable spinal motor neurons. We have published (Mekhael 2019) the first direct link between overexpression of a specific neuronal co-transporter and the emergence of spasticity, another condition associated with motor neuron hyperexcitability. This Na-K-Cl cotransporter (NKCC1) is found on motor neurons and is involved in maintaining chloride gradient. We reported that our non-invasive intervention suppresses NKCC1 levels and reduces spasticity. We have been applying our approach to ALS and have established the SOD1-G93A mouse model at our lab. Initial studies with these mice show that stimulation with our multi-site DCS technology results in reduction of tremor, slowing of disease progression, increased survival and improvement of motor function. We have characterized the effects of stimulation at a molecular level, and have found that stimulation with our technology increases HSP70 protein expression levels, and decreases SOD1 protein expression levels. Our MyoRegulator platform incorporates our multi-site DCS technology for human use, and has now completed two clinical trials in spasticity (post-stroke), with a positive readout in both trials, thereby demonstrating safety and efficacy.

Pipeline
We are focused on advancing two first-in-class products based on our proprietary multi-site DCS technology, and these represent the first neuromodulation devices for treating Spasticity and for treating ALS. Based on our foundational patent portfolio, PathMaker is developing an innovative pipeline of neuromodulation products for treating serious neurological disorders:

MyoRegulator® is our company's™ first product, and provides a breakthrough non-invasive treatment for muscle spasticity. Spasticity is a common condition seen in many patients suffering from stroke, ALS, cerebral palsy, multiple sclerosis, spinal cord injury and traumatic brain injury. MyoRegulator is based on our proprietary DoubleStim® technology, which provides simultaneous non-invasive stimulation at spinal and peripheral sites, resulting in suppression of hyperexcitable spinal circuits found in patients with spasticity.

ALSuppressor is our company's second product, and is the first neuromodulation device intended for the treatment of ALS. Important links between ALS and motor neuron hyperexcitability have been documented, and our published work in NKCC1 suppression has led us to the application of our technology to ALS. ALSuppressor has emerged from our proprietary DoubleStim® technology, and provides simultaneous non-invasive stimulation at multiple spinal and peripheral sites, as well as bilateral stimulation.

IP Status
PathMaker Neurosystems Inc. holds an exclusive, worldwide, life-of-patent-family license from the City University of New York (CUNY) relating to the core MyoRegulator technology and extensions thereof and covering PathMaker implementations, including the MyoRegulator device and its application to ALS. 46 issued US and foreign patents protect MyoRegulator to date, including issued patents already covering this device in US, EU, Japan, China, Israel, Singapore and Mexico. A number of patents have already been filed covering application of our proprietary approach to the treatment of ALS and related diseases. We have done freedom to operate analysis and it came back clean. We have registered trademarks protecting DoubleStim and MyoRegulator trademarks in both US and EU. There is no other neuromodulation device at present that treats spasticity or ALS, and MyoRegulator is expected to be the first.

Goals
Our goals are to build awareness for our company and of our novel approach to treating ALS using neuronal hyperexcitability suppression. We are working on an equity financing for commercial launch of our first product, and also looking to identify strategic partners for our products – please reach out for those interested.

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CEO and Founder

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Company Profile

Pyrinovo

Stage
Early/startup

Indication

ALS, FTD, Alzheimer's (any disease of TDP43 mislocalization)

Therapeutic Platform

CHMP7/ESCRT3 pathway. Inhibition/loss of CHMP7 repairs nuclear pore dysfunction and subsequent TDP43 loss of function. This makes it an "upstream" therapy for (95% of sporadic ALS, FTD and 50% of Alzheimer's disease).

Pipeline

Antisense oligonucleotide, already developed, now undergoing safety testing. Validated efficacy in Sporadic iPS cell lines (>30 different patients) C9orf72 iPS lines (>10 different patients)
To date the ASO is safe and provides highly specific knockdown in vivo ((mice and human spinal iPS cell lines) Already tested in >30 different sporadic ALS iPS spinal cord preparations.
Additional candidate therapies: ShRNA, Trim Away protein degeneration of CHMP7.
To be developed: small molecular inhibitor.

IP Status

Under review with PTO.
First in class drug; No competing therapy/technology

Goals

Startup funding (requires brinks and motor NewCo, CEO, and CSO)

Contact Name

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Founder

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Company Profile

RAYA THERAPEUTIC
A CURE FOR ALS

Raya Therapeutic, Inc

Stage
P3 with multiple compounds in 2H/2023

Indication
Primary Focus ALS; also potential in two ultra-rare pediatric indications (P3 2024).

Therapeutic Platform
Raya is perhaps the only company in the industry that planning to develop five clinical stage compounds each with different mechanisms of action (MOA) targeting ALS. In addition to testing the drugs by themselves, the company is planning to test them all in combination. I vitro (iPSC MN) and in vivo (SOD1 mouse) studies have already started to test various combinations to look for synergistic effects. 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.

Molecule	Molecule support in ALS models	Target support in ALS models	Target support in neurodegeneration models	Clinical Insights
RT1978	n/a	++	+++	Orally bioavailable BBB-penetrant Good safety profile
RT2010	n/a	+++	+++	Orally bioavailable BBB-penetrant Good safety profile
RT1999	(+++)	(+++)	(+++)	Orally bioavailable BBB-penetrant Good safety profile Open IND; ODD in ALS in both US &EU
RT1972	+	++	+++	Orally bioavailable BBB-penetrant Good safety profile
RT1968	++	+++	+++	Good safety profile BBB-penetrant

+ 1 published study ++ a few published studies +++ 5+ published studies () unpublished studies

IP Status
3 of the 5 compounds have existing IP. All will be eligible for NCE and ODD exclusivity of 5 and 7 years respectively (USA).

Raya will be filing new IP on all the drugs in the coming months.

Goals
Raya is currently raising a Series B round of $15-25M to get all five compounds ready to start a P3 trial (single, dedicated platform design).

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Founder

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Company Profile

Satellos bioscience

Satellos Bioscience, Inc

Stage
Pre-Clinical

Indications

Duchenne Muscular Dystrophy

Therapeutic Platform

The MyoReGenX™ platform is unique to Satellos. Grounded in decades of pioneering muscle stem cell knowledge established by the Rudnicki lab, it serves three core functions: 1) Recapitulation of the muscle stem cell micro-environment (the "niche"); 2) Identification, classification and confirmation of regenerative deficits in serious muscle diseases; and, 3) Discovery and development of therapeutic solutions for those cases where regeneration is found to be impaired. MyoReGenX™ was specifically designed to recreate the specialized muscle stem cell niche in vitro. Maintaining this micro-environment is an absolute sina qua non for sustaining the identity and function of muscle stem cells outside of the body. MyoReGenX™ uniquely enables us to: map pathways which modulate muscle repair and regeneration, identify and confirm drug targets, and test drug candidates for their ability to restore muscle stem cell repair mechanisms that have been impaired by genetic mutations, disease or injury. Deploying MyoReGenX™, Satellos is developing novel muscle stem cell targeted regenerative therapeutics with the potential to greatly improve the lives of individuals suffering from serious muscle diseases. Our initial focus areas are muscular dystrophies and aging muscle.

Pipeline

Duchenne Muscular Dystrophy (Preclinical) MDC1A (Discovery) ColVI Myopathies (Discovery)

IP Status

Satellos holds a number of issued and pending patents related to therapeutics and their use in regenerating skeletal muscle through the targeted regulation of muscle stem cells. We are building a portfolio of patents surrounding the notion of stem cell polarity modulation, as well as composition of matter patents pertaining to molecules capable of regulating this process for the purpose of treating certain muscular diseases.

Goals for Presentation

To increase awareness surrounding our innovative approach to restoring impaired regeneration in Duchenne through regulation of muscle stem cell polarity.

Presenter Name

Ryan Mitchell, PhD
Director of Business Development

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Company Profile

University of Pittsburgh

University of Pittsburgh

Stage
Grants/Stipends

Indications

LEMS, SMA, ALS, BOTOX poisoning, old age-induced frailty (dynapenia)

Therapeutic Platform
Small molecule

Pipeline
We are looking for funding to further develop our Cav2 calcium channel gating modifier

IP Status
Patent protection in the USA only for the current lead molecule

Goals for Presentation
To present the background and preclinical data to support the development of a small molecule Cav2 calcium channel gating modifier as a symptomatic treatment for a variety of neuromuscular weakness diseases and conditions that have a presynaptic transmitter release deficit.

Presenter Name
Stephen Meriney, PhD
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