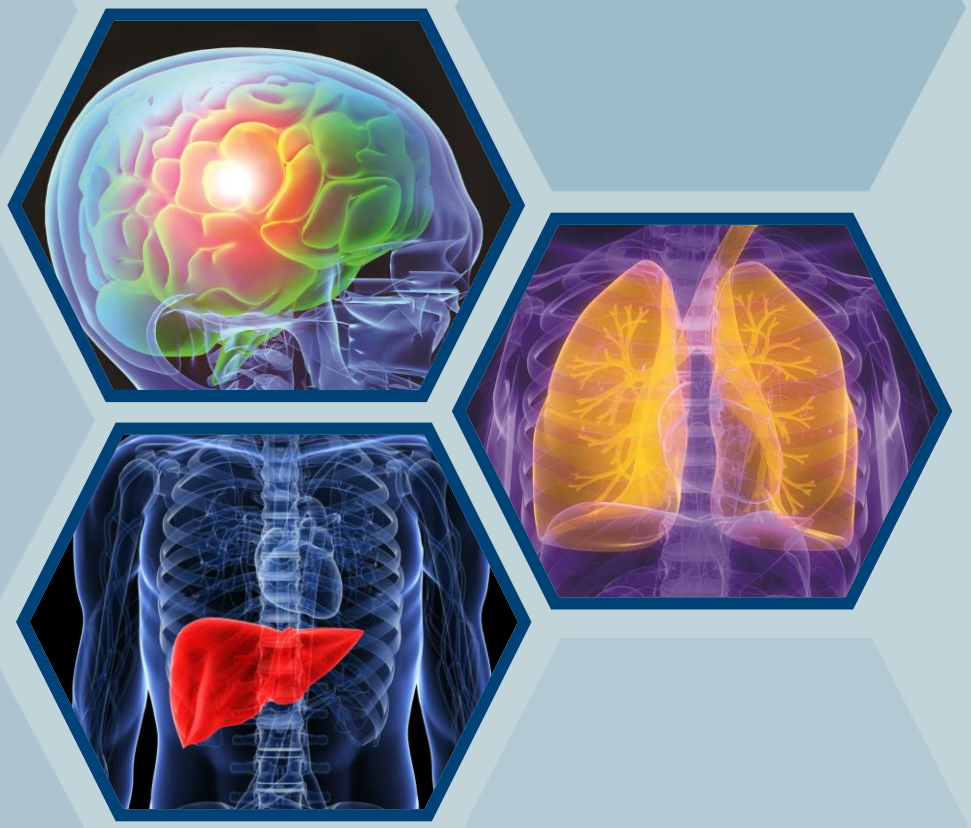

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Corporate Presentation

Forward-Looking Statements

Statements in this presentation that are not historical in nature constitute forward-looking statements within the meaning of the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. These forward-looking statements include statements regarding MediciNova's clinical trials supporting the safety and efficacy of its product candidates and the potential novelty of such product candidates as treatments for disease, plans and objectives for clinical trials and product development, strategies, future performance, expectations, assumptions, financial condition, liquidity and capital resources. These forward-looking statements include, without limitation, statements regarding the future development and efficacy of MN-166, MN-221, MN-001, and MN-029. These forward-looking statements may be preceded by, followed by or otherwise include the words "believes," "expects," "anticipates," "intends," "estimates," "projects," "can," "could," "may," "will," "would," "considering," "planning" or similar expressions. These forward-looking statements involve a number of risks and uncertainties that may cause actual results or events to differ materially from those expressed or implied by such forward-looking statements. Factors that may cause actual results or events to differ materially from those expressed or implied by these forward-looking statements include, but are not limited to, risks of obtaining future partner or grant funding for development of MN-166, MN-221, MN-001, and MN-029 and risks of raising sufficient capital when needed to fund MediciNova's operations and contribution to clinical development, risks and uncertainties inherent in clinical trials, including the potential cost, expected timing and risks associated with clinical trials designed to meet FDA guidance and the viability of further development considering these factors, product development and commercialization risks (including reliance on a joint venture entity in China to develop and commercialize MediciNova's product candidates in China), risks related to MediciNova's reliance on the success of its MN-166 and MN-001 product candidates, the uncertainty of whether the results of clinical trials will be predictive of results in later stages of product development, the risk of delays or failure to obtain or maintain regulatory approval, risks associated with the reliance on third parties to sponsor and fund clinical trials, risks regarding intellectual property rights in product candidates and the ability to defend and enforce such intellectual property rights, the risk of failure of the third parties upon whom MediciNova relies to conduct its clinical trials and manufacture its product candidates to perform as expected, the risk of increased cost and delays due to delays in the commencement, enrollment, completion or analysis of clinical trials or significant issues regarding the adequacy of clinical trial designs or the execution of clinical trials, and the timing of expected filings with the regulatory authorities, MediciNova's collaborations with third parties, the availability of funds to complete product development plans and MediciNova's ability to obtain third party funding for programs and raise sufficient capital when needed, and the other risks and uncertainties described in MediciNova's filings with the Securities and Exchange Commission, including its annual report on Form 10-K for the year ended December 31, 2018 and its subsequent periodic reports on Forms 10-Q and 8-K. Undue reliance should not be placed on these forward-looking statements, which speak only as of November 14, 2019. MediciNova disclaims any intent or obligation to revise or update these forward-looking statements.

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MediciNova Overview

MediciNova, Inc. is a publicly-traded, development-stage biopharmaceutical company **focused on acquiring and developing novel, small-molecule therapeutics for the treatment of diseases with unmet medical needs**.

Headquarters	Dual-Listed
La Jolla, California	Listed in both the U.S. and Japan NASDAQ: MNOV TSE - JASDAQ: 4875

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Investment Highlights

Novel product candidates in clinical development with encouraging efficacy and safety data	
MN-166 (ibudilast)	Treatment of Neurological Diseases i.e. Progressive MS, ALS, Cervical Myelopathy, Peripheral Neuropathy, Glioblastoma, and Substance Dependence • Approved in Japan in 1989 - post-stroke dizziness and asthma • Large safety database
MN-001 (tipelukast)	Treatment of Fibrotic Diseases i.e. IPF (idiopathic pulmonary fibrosis) Treatment of Hyperlipidemia and Fibrotic Disease i.e. NASH (nonalcoholic steatohepatitis) and NAFLD (nonalcoholic fatty liver disease)
Well capitalized	
Experienced management team	

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Programs in Clinical Development

Core programs/ Indications	Status	Preclinical	Phase 1	Phase 2	Phase 3
MN-166, Oral Anti-Inflammatory / Neuroprotective Therapeutic					
NEURODEGENERATIVE DISEASES					
Progressive Multiple Sclerosis NeuroNEXT / Cleveland Clinic (Funded by NINDS)	FAST TRACK	Completed Phase 2			
ALS (Amyotrophic Lateral Sclerosis) ★ Carolinas / Massachusetts General Hospital (MGH)	FAST TRACK	Phase 3 trial ongoing			
Degenerative Cervical Myelopathy (DCM) University of Cambridge (Funded by NIHR in the UK)		Ongoing			
Chemotherapy-Induced Peripheral Neuropathy (CIPN) University of Sydney (Funded by Concord Cancer Centre)		Ongoing			
Glioblastoma (GBM) ★ Dana-Farber Cancer Institute		Ongoing			
SUBSTANCE DEPENDENCE					
Methamphetamine Dependence UCLA / Oregon Health & Science (Funded by NIDA / VA)	FAST TRACK	Completed UCLA trial / OHSU trial ongoing			
Opioid Dependence Columbia University (Funded by NIDA)		Completed Phase 2 trial			
Alcohol Dependence UCLA (Funded by NIAAA / NIDA)		Completed one trial / Two trials ongoing			
MN-001, Oral Anti-Inflammatory / Anti-Fibrotic Therapeutic					
NASH (Nonalcoholic Steatohepatitis) / NAFLD	FAST TRACK	Completed Phase 2 trial			
IPF (Idiopathic Pulmonary Fibrosis) ★	FAST TRACK	Ongoing			

★ *Orphan Drug*

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Developing Novel Therapeutics

MN-166

Ibudilast

MN-001

Tipelukast

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MN-166

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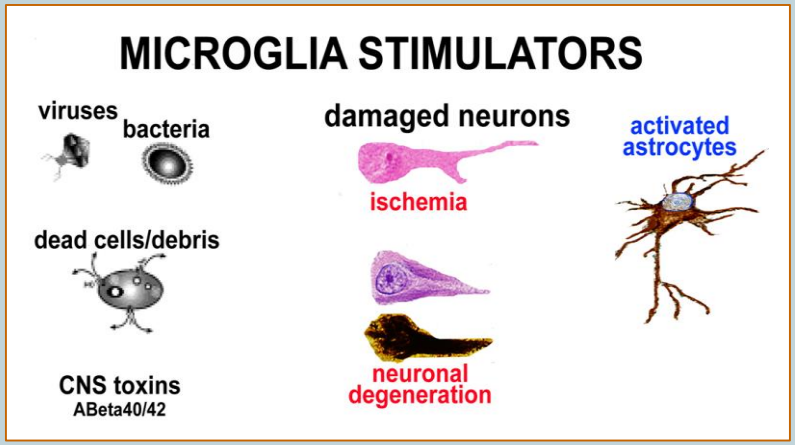
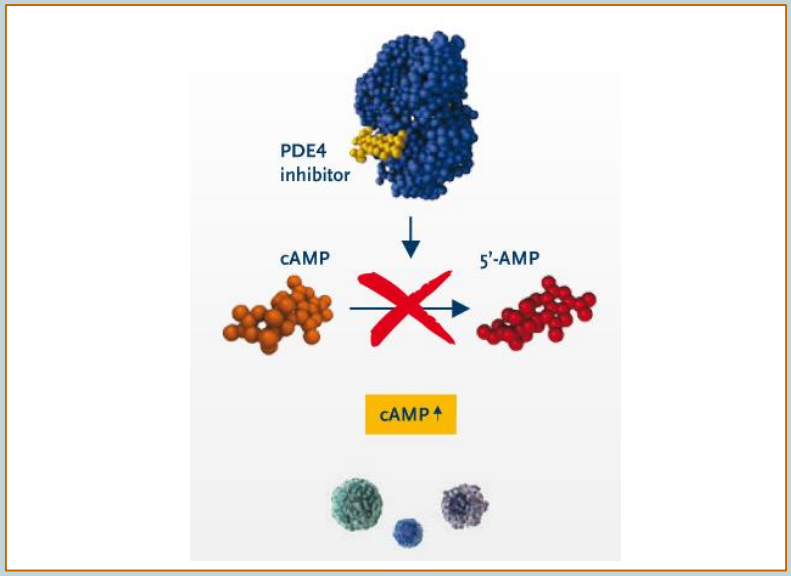
How does MN-166 work?





MIF Inhibition

- Linked to attenuated disease progression in animal models of MS

PDE 4 Inhibition

- Increases cAMP
- Reduces pro-inflammatory cytokines (i.e. IL-1, TNF-α, IL-6)
- Neuroprotection

GLIAL CELL ATTENUATION

- Role of Glia:
  - Type of macrophage
  - Activated during brain damage
  - **Glial activation leads to neurodegeneration**

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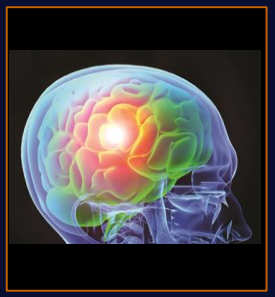

Progressive Multiple Sclerosis (MS)

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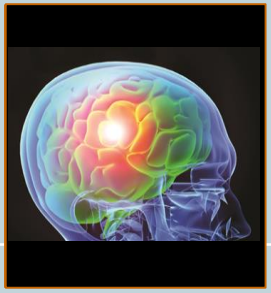
Progressive Multiple Sclerosis (MS)



MS AFFECTS

2.3M

Worldwide[1]

400,000

in United States[1]

DIMINISHED QUALITY OF LIFE

(e.g. fatigue, walking difficulties, weakness, pain, cognitive changes, depression)[1]

CURRENT MS MARKET*

$21.5B

Worldwide

(*$19.1B out of $21.5B total sales in 2018 were for drugs approved for RRMS only.)

SPMS w/o Relapses:

NO APPROVED DRUGS

for long-term treatment of SPMS without Relapse

SPMS with Relapses:

MAYZENT (siponimod)

MAVENCLAD (cladribine)[2]

PPMS:

OCREVUS (ocrelizumab)

for Primary Progressive MS

Large Market Opportunity for Secondary Progressive MS (SPMS) without Relapses

- The vast majority of secondary progressive MS patients do not have relapses.
- Only 10.0% of placebo-treated SPMS subjects had a relapse during the Phase 2b trial of MN-166 (ibudilast) over 96 weeks of treatment.
- Only 18.7% of placebo-treated SPMS subjects had a relapse during the Phase 3 trial of MAYZENT (siponimod) with a median study duration of 21 months.

1. *Source: National Multiple Sclerosis Society*
2. *MAVENCLAD's Prescribing Information has a Boxed Warning for an increased risk of malignancy and fetal harm. It is generally recommended for patients who have had an inadequate response to, or are unable to tolerate, an alternate MS drug.*

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MN-166 Phase 2b Progressive MS Trial

Completed

SPRINT-MS: Phase 2b Trial in Progressive MS (Completed)	
FUNDING	Funded by NIH grant through NINDS
PRIORITY	**Ibudilast was the first drug chosen by NINDS for an interventional clinical trial in the NeuroNEXT program**
PRINCIPAL INVESTIGATOR	**Robert Fox, M.D.** Cleveland Clinic
CLINICAL COORDINATING CENTER	Massachusetts General Hospital
DATA COORDINATING CENTER	University of Iowa
SITES	28 academic medical centers in the NeuroNEXT network
ADDITIONAL FUNDING	National Multiple Sclerosis Society provided patient advocate input and trial enrollment awareness and also provided additional funding

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MN-166 Phase 2b Progressive MS Trial

Completed

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SPRINT-MS: Trial Design	
TRIAL DESIGN	N = 255 subjects with Primary or Secondary Progressive MS (PPMS or SPMS) Interferon-beta or glatiramer acetate are allowed as concomitant medication
	Phase 2b randomized, double-blind trial; 96-weeks; 28 centers in the U.S. (NeuroNEXT sites)
	Dosing: up to 100 mg/day (50 mg BID) of MN-166 (ibudilast) or placebo (1:1 randomization)
OBJECTIVES	Primary Endpoint #1: whole brain atrophy using brain parenchymal fraction (BPF) Primary Endpoint #2: safety and tolerability
	Secondary: disability, imaging analyses of brain and retinal tissue integrity, cortical atrophy, cognitive impairment, quality-of-life, and neuropathic pain
STATUS	• Completed • Top-line data was presented at ECTRIMS conference • Disability data was presented at ACTRIMS conference • Results published in the New England Journal of Medicine

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MN-166 Phase 2b Progressive MS Trial Sites

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Albert Einstein College of Medicine	University of California - Davis
Brigham and Women's Hospital	University of California - Los Angeles
Cleveland Clinic	University of Cincinnati
Columbia University Medical Center	University of Colorado – Denver
Emory University	University of Kansas Medical Center
Massachusetts General Hospital	University of Miami School of Medicine
Northwestern University	University of Pittsburgh
Ohio State University	University of Rochester
Oregon Health and Science University	University of Texas Southwestern
SUNY Buffalo	University of Utah
SUNY Stony Brook	University of Virginia – Charlottesville
SUNY Upstate	Vanderbilt University
Swedish Medical Center - Seattle	Washington University in St. Louis
University of Alabama at Birmingham	Weill Cornell Medical College

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MN-166 Phase 2b Progressive MS Trial

Completed

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SPRINT-MS: Results	
ACHIEVED PRIMARY ENDPOINT #1: BRAIN ATROPHY	• MN-166 (ibudilast) demonstrated a statistically significant **48% reduction in the rate of progression of whole brain atrophy vs. placebo (p=0.04)** as measured by MRI analysis using brain parenchymal fraction (BPF).
ACHIEVED PRIMARY ENDPOINT #2: SAFETY AND TOLERABILITY	• MN-166 (ibudilast) demonstrated a **favorable safety and tolerability profile**. • No increased rate of serious adverse events in the MN-166 (ibudilast) group compared to the placebo group. • No opportunistic infections, no cancers, no cardiovascular events (no heart attacks or strokes), and no deaths related to MN-166 (ibudilast) treatment.
	• No statistically significant difference in tolerability between the MN-166 (ibudilast) group and the placebo group. • The most common treatment-emergent adverse events during the study were gastrointestinal adverse events, which occurred with a higher frequency in the MN-166 (ibudilast) group, and upper respiratory tract infections, which occurred with a higher frequency in the placebo group.
DISABILITY PROGRESSION	• MN-166 (ibudilast) demonstrated a 26% reduction in the risk of confirmed disability progression vs. placebo (hazard ratio = 0.74), measured by EDSS.

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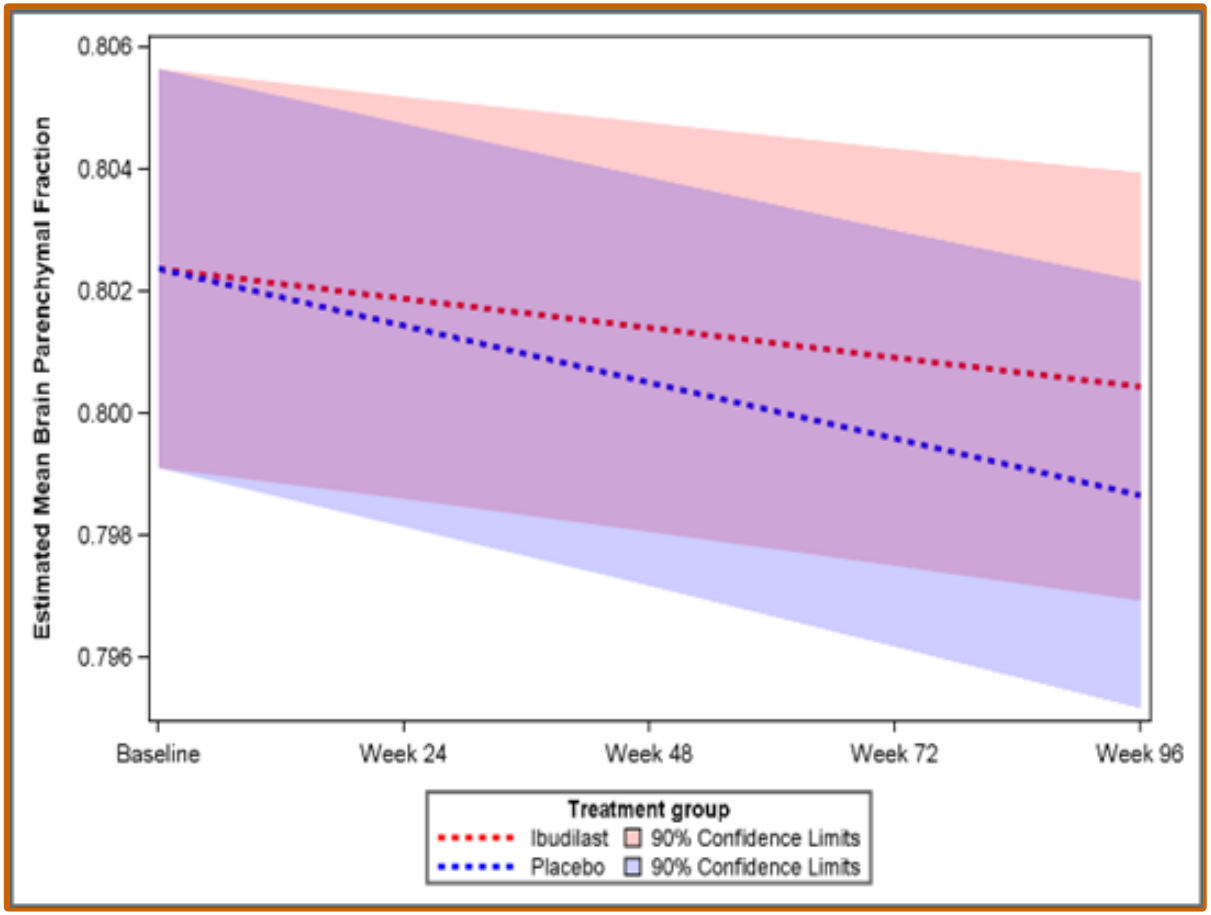
MN-166 Phase 2b Progressive MS Trial

Completed

Ibudilast Reduced Brain Atrophy Progression by 48% (p=0.04)



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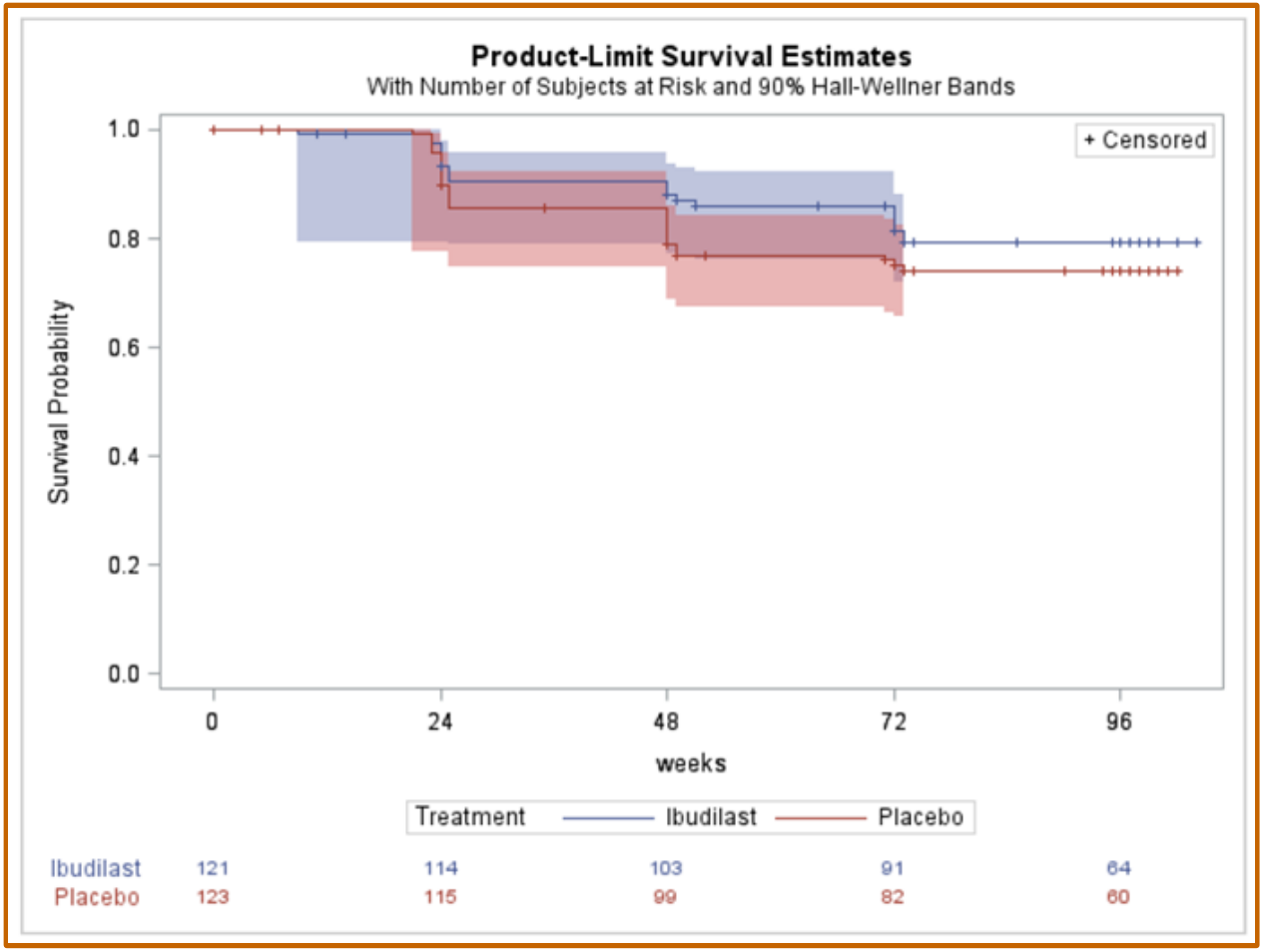
MN-166 Phase 2b Progressive MS Trial

Completed

Ibudilast Reduced the Risk of Confirmed Disability Progression by 26%*



** Hazard ratio = 0.74, Confirmed disability progression was measured using EDSS*

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MN-166 Phase 2b Progressive MS Trial

Completed

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Risk of Confirmed Disability Progression by Subgroup				
Subgroup	Number of Subjects MN-166	Number of Subjects Placebo	Hazard Ratio*	Risk Reduction
Primary Progressive MS	68	66	0.707	29%
Secondary Progressive MS with Relapse	9	6	1.153	-15%
Secondary Progressive MS without Relapse	52	54	0.538	46%

* MN-166 vs. Placebo

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MN-166 Phase 2b Progressive MS Trial

Completed

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We Believe MN-166 (ibudilast) has Potential to be the Best-in-Disease Drug for Progressive MS					
Drug	Type of Progressive MS	Route of Administration	Phase / Study Size	Reduction in Brain Atrophy after 2 Years	Reduction in Disability Progression
ocrelizumab	PPMS	intravenous infusion	Phase 3 n=732	17.5%	24%
siponimod	SPMS	oral	Phase 3 n=1651	15%	21%
MN-166	**PPMS and SPMS**	**oral**	**Phase 2b n=255**	**48%**	**PPMS: 29%** **SPMS without Relapse: 46%**

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MN-166 Phase 2b Progressive MS Trial

Completed

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We Believe MN-166 (ibudilast) has Potential to be the Best-in-Disease Drug for Progressive MS		
Drug	**Safety Issues**	**Most Common Adverse Reactions**
ocrelizumab (OCREVUS)	• malignancies including breast cancer • serious infusion reactions • infections	• upper respiratory tract infections • infusion reactions • skin infections • lower respiratory tract infections
siponimod (MAYZENT)*	• infections • macular edema • bradyarrhythmia • respiratory effects • liver injury • increased blood pressure • fetal risk	• headache • hypertension • transaminase increased • falls • edema peripheral
MN-166	• **None**	• **gastrointestinal side effects**

** MAYZENT requires 7 assessments prior to first dose: CYP2C9 Genotype Determination, Complete Blood Count, Ophthalmic Evaluation, Cardiac Evaluation, Current or Prior Medications, Vaccinations, and Liver Function Tests.*

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MN-166 Progressive MS Phase 3 Plan

Progressive MS Phase 3 Trial Plan

Phase 3 Trial will enroll only subjects with SPMS without relapses

- FDA agreed that SPMS without relapses is an appropriate target population
- Based on the subgroup analysis, MediciNova believes that subjects with SPMS without relapses will have the best response to MN-166 treatment
- The unmet medical need is highest in subjects with SPMS without relapses
  - No drugs approved for long-term treatment of SPMS without relapses
  - It is the largest subgroup of progressive MS patients (>80% of SPMS patients)

FDA agreed that the primary endpoint should be time to 3-month confirmed disability progression, as measured by EDSS

MediciNova plans to conduct a single Phase 3 trial

- FDA acknowledged that a single trial can be the basis for marketing approval
- FDA approved both MAYZENT and MAVENCLAD for relapsing SPMS in March 2019 after a single Phase 3 trial for each drug

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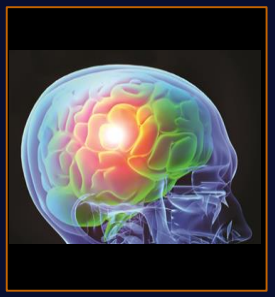

Amyotrophic Lateral Sclerosis (ALS)

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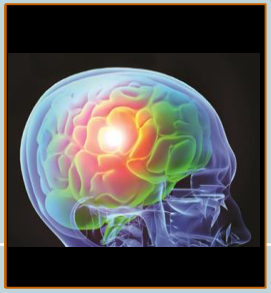
Amyotrophic Lateral Sclerosis (ALS)
“Lou Gehrig's Disease”



ALS AFFECTS

~20,000

People
in United States[1]

ORPHAN INDICATION

LIFE EXPECTANCY

2-5 YRS[1]

FATAL

An effective new drug for ALS could generate sales of

$1B+[2]

EXPECTED MARKET OPPORTUNITY

APPROVED DRUGS

RILUZOLE

Increases survival by ONLY 2-3 months[3]

RADICAVA

inconvenient IV infusion; hit ALSFRS-R endpoint; disease duration ≤2 years[4]

1. *Source: ALS Association*
2. *Source: Cowen & Co. estimate*
3. *Cochrane Database of Systematic Reviews*
4. *Radicava prescribing information*

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MN-166 Phase 2 ALS Trial

Completed

ALS Phase 2 Trial Design	
TRIAL DESIGN	N = 51 ALS subjects not using non-invasive ventilation
	Phase 2 randomized, double-blind trial at Carolinas Neuromuscular/ALS-MDA Center Principal Investigator: Dr. Benjamin Rix Brooks
	Duration: 6 months of double-blind treatment + open label extension (6 months)
	Dosing: 60 mg/day of MN-166 or placebo (2:1 randomization) with riluzole
OBJECTIVES	Primary endpoint: safety and tolerability Other endpoints: functional activity (ALSFRS-R), respiratory function, muscle strength, quality of life, Clinical Global Impression of Change, serum creatinine as a biomarker, and pharmacokinetics
STATUS	• Completed • Top-line data presented at the International Symposium on ALS/MND • Received FDA feedback on pivotal trial design in September 2018

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MN-166 Phase 2 ALS Trial

Completed

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ALS Trial: Top-Line Results	
ACHIEVED PRIMARY ENDPOINT: SAFETY AND TOLERABILITY	• MN-166 (ibudilast) demonstrated a **favorable safety and tolerability profile**. • 7 serious adverse events (SAEs) but none were related to the study drug • All treatment-related adverse events (TRAEs) were mild to moderate • No severe or life-threatening TRAEs • Most frequently reported TRAEs: nausea, anorexia, and loss of appetite were expected and are common side effects of both riluzole and MN-166 (ibudilast)
EFFICACY TRENDS: ALSFRS-R RESPONDERS	• Responder was defined as a subject who improved on the ALSFRS-R total score*, had no change on the score, or the score declined by 1 point
	• 6-month, double-blind period: 29.4% of subjects in the MN-166 (ibudilast) group were responders compared to 17.6% of subjects in the placebo group
	• 6-month, open-label extension (OLE): 35.3% of subjects on placebo in the double-blind period were responders when taking MN-166 (ibudilast) in OLE

** Amyotrophic Lateral Sclerosis Functional Rating Scale-Revised (ALSFRS-R) total score measures the functional activity of an ALS subject. ALS subjects decline on the ALSFRS-R total score over time as the disease progresses and their symptoms worsen.*

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MN-166 Degenerative Cervical Myelopathy (DCM) Trial

Ongoing

MN-166

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Degenerative Cervical Myelopathy (DCM) Trial Design	
TRIAL DESIGN	N = 362 subjects with degenerative cervical myelopathy (DCM) who are scheduled for first surgical decompression (including enrollment of 25-80 in the pilot stage)
	Phase 3 randomized, double-blind, multicenter trial Principal Investigator: Dr. Mark Kotter, University of Cambridge
	Duration: 8 months of double-blind treatment + follow up (6 months)
	Dosing: up to 100 mg/day of MN-166 or placebo (1:1 randomization)
OBJECTIVE	Primary endpoints: (1) modified Japanese Orthopaedic Association (mJOA) Score (evaluates motor dysfunction in upper and lower extremities, loss of sensation, and bladder sphincter dysfunction) at 6 months after surgery; (2) Visual Analogue Scale (VAS) neck pain at 6 months after surgery
STATUS	Ongoing (announced kick-off meeting in May 2019)

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MN-166 Chemotherapy-Induced Peripheral Neuropathy (CIPN) Trial

Ongoing

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Chemotherapy-Induced Peripheral Neuropathy (CIPN) Trial Design	
TRIAL DESIGN	N = 20 subjects with metastatic gastrointestinal cancer (colorectal cancer and upper gastrointestinal cancers) who are receiving oxaliplatin
	Open-label, sequential cross-over clinical study at the University of Sydney Concord Cancer Centre in Australia Principal Investigator: Dr. Janette Vardy
	Duration: 3 months
	Dosing: 1) one cycle of chemotherapy without MN-166, followed by 2) one cycle of chemotherapy with 30 mg MN-166 twice daily
OBJECTIVES	Determine the effect of MN-166 on: 1) the development of acute neurotoxicity 2) the severity of chemotherapy-induced peripheral neuropathy (CIPN); and 3) pharmacokinetics of oxaliplatin and fluorouracil
STATUS	Ongoing (announced initiation of study in March 2018)

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MN-166 Glioblastoma (GBM) Trial

Ongoing

MN-166

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Glioblastoma (GBM) Trial Design	
TRIAL DESIGN	Part 1: N = 15 - 18 subjects with recurrent glioblastoma Part 2: N = 32 subjects with recurrent glioblastoma
	Open-label clinical study at Dana-Farber Cancer Institute (DFCI) in Boston Principal Investigators: Patrick Wen, M.D., Harvard Medical School / DFCI Kerrie McDonald, Ph.D., Lowy Cancer Research Centre, UNSW Australia
	Duration: 28 days for Part 1; 6 months for Part 2
	Dosing: Part 1: MN-166 dose-escalation 60-100 mg/day + temozolomide Part 2: MN-166 fixed-dose + temozolomide
OBJECTIVES	1) Safety and tolerability 2) Proportion of patients who are progression-free at 6 months Other objectives: overall survival; response rate; median 6-month progression-free survival
STATUS	Ongoing (announced initiation of study in January 2019)

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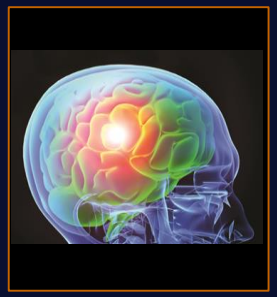

Substance Dependence and Addiction

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MN-166
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MN-166: Opioid Dependence

Summary of MN-166 Opioid Dependence Studies and Data	
Opioid Withdrawal & Analgesia Phase 1b/2a Trial (COMPLETED)	**MN-166 Reduced Subjective Opioid Withdrawal Scale (SOWS)** • MN-166 significantly reduced perspiring ($p<0.05$) and hot flashes ($p<0.05$), two components of SOWS • Principal Investigator: Dr. Sandra Comer, Columbia University
Opioid Self-Administration Phase 2 Trial (COMPLETED)	MN-166 significantly **decreased the craving** for • heroin ($p<0.01$), • cocaine ($p<0.01$) • tobacco ($p<0.05$)
	MN-166 significantly **decreased the reinforcing effects of oxycodone** ($p<0.05$)
	MN-166 significantly **enhanced the analgesic effects of oxycodone** ($p<0.05$)
	Principal Investigator: Dr. Sandra Comer, Columbia University

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MN-166: Methamphetamine Dependence

MN-166
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Summary of MN-166 Methamphetamine Dependence Studies and Data	
Methamphetamine Dependence Phase Ib Trial (COMPLETED)	• MN-166 significantly reduced perseverations (p=0.01) and variability in response times (p=0.006), suggesting a protective effect on sustained attention • Principal Investigators: Dr. Steven Shoptaw and Dr. Keith Heinzerling, University of California, Los Angeles (UCLA)
Methamphetamine Dependence Phase 2 Trial (COMPLETED)	• Phase 2 randomized, double-blind, placebo-controlled outpatient study in methamphetamine-dependent subjects • Did not achieve the primary endpoint of abstinence during the final two weeks of treatment • Principal Investigator: Dr. Keith Heinzerling, UCLA
Methamphetamine Dependence Phase 2 Trial (ONGOING)	• Ongoing Phase 2 randomized, double-blind, placebo-controlled study in recently-abstinent methamphetamine users • Endpoints include effects of MN-166 on neuroinflammation, brain function, and methamphetamine craving • Principal Investigator: Dr. Milky Kohno, Oregon Health & Science University

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MN-166: Alcohol Dependence

Summary of MN-166 Alcohol Dependence Studies and Data	
Alcohol Dependence Phase 2a Trial (COMPLETED)	• MN-166 significantly decreased basal, daily alcohol craving over the course of the study ($p<0.05$) • Principal Investigator: Dr. Lara Ray, UCLA
Alcohol Dependence and Withdrawal Phase 2 Trial (ONGOING)	• Ongoing Phase 2, randomized, double-blind, placebo-controlled, outpatient trial in up to 50 non treatment-seeking individuals with moderate-to-severe alcohol use disorder • Primary Endpoints: determine whether MN-166 reduces basal level negative affect during abstinence and interferes with alcohol-induced blunting of negative affectivity • Principal Investigator: Dr. Lara Ray, UCLA
Alcohol Dependence Phase 2b Trial (ONGOING)	• Ongoing Phase 2b, randomized, double-blind, placebo-controlled, outpatient trial in up to 132 treatment-seeking individuals with moderate or severe alcohol use disorder • Primary Endpoint: determine whether MN-166 will decrease percent heavy drinking days (≥ 5 drinks for men / ≥ 4 drinks for women) • Principal Investigator: Dr. Lara Ray, UCLA

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Fibrosis

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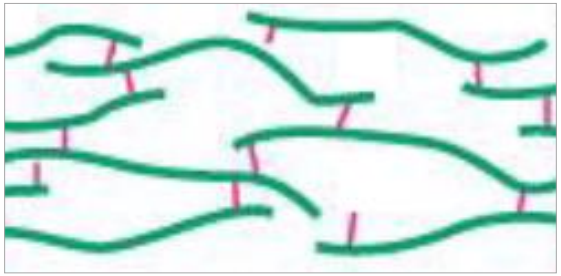
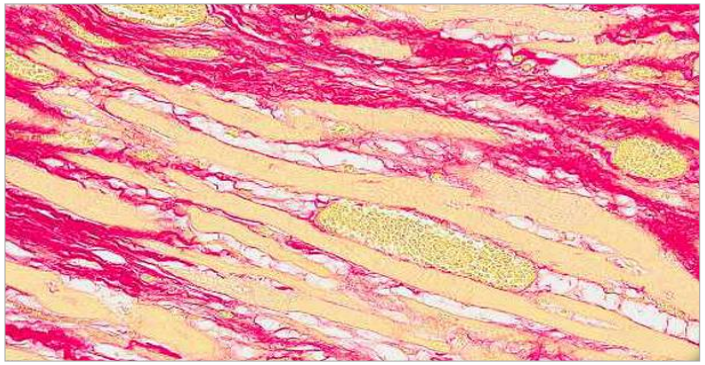
What is Fibrosis?

CROSS-LINKING OF COLLAGEN AND ELASTIN



FIBROSIS



- Fibrosis is the development of excess fibrous connective tissue in an organ
- Fibrosis is a result of inflammation, irritation, or healing (e.g. scar)
- Cross-linking of collagen and elastin is the final step in fibrosis development

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MN-001

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How does MN-001 work?

Anti-fibrotic Activity

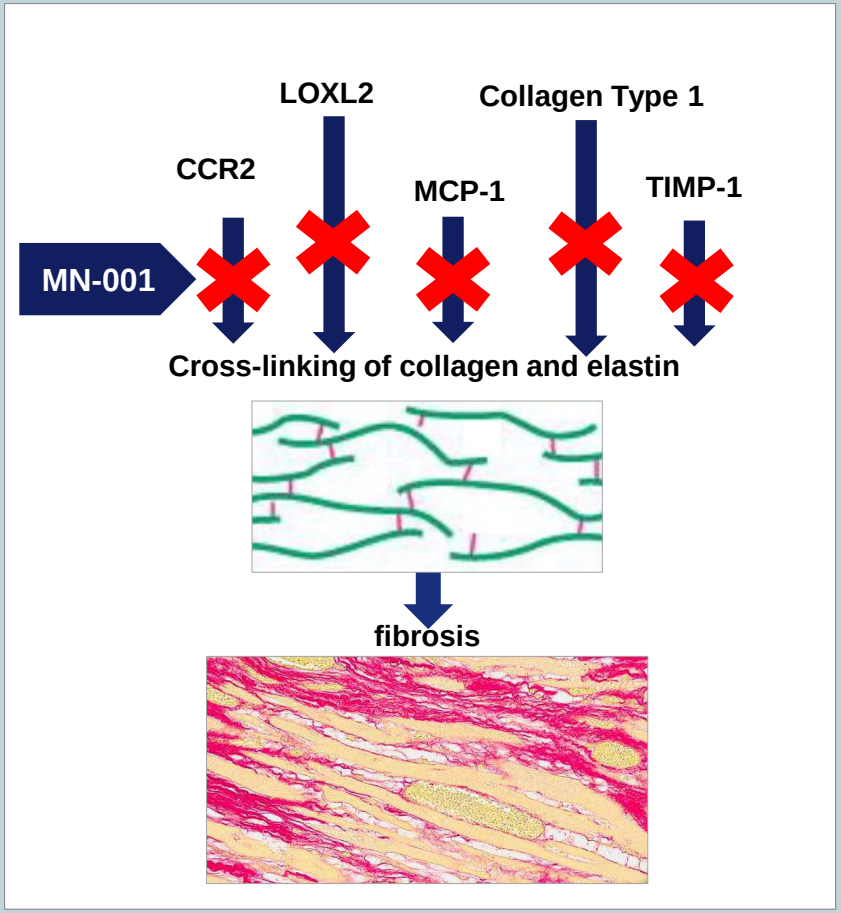
- MN-001 Reduces mRNA expression of genes that are known to promote fibrosis (e.g. LOXL2, Collagen Type 1, TIMP-1)
- MN-001 Inhibits 5-lipoxygenase (5-LO)

Anti-inflammatory Activity

- MN-001 Inhibits leukotriene (LT) and phosphodiesterases (PDE)
- MN-001 Reduces inflammatory gene expression (e.g. CCR2, MCP-1)

Reduces Triglycerides

- MN-001 Reduced triglycerides in every clinical trial completed (asthma, interstitial cystitis)



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MN-001 Background

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More than 600 human subjects exposed to MN-001 in prior studies

- Completed Phase 2 study of MN-001 in asthma with positive results

- MN-001 was considered generally safe and well-tolerated

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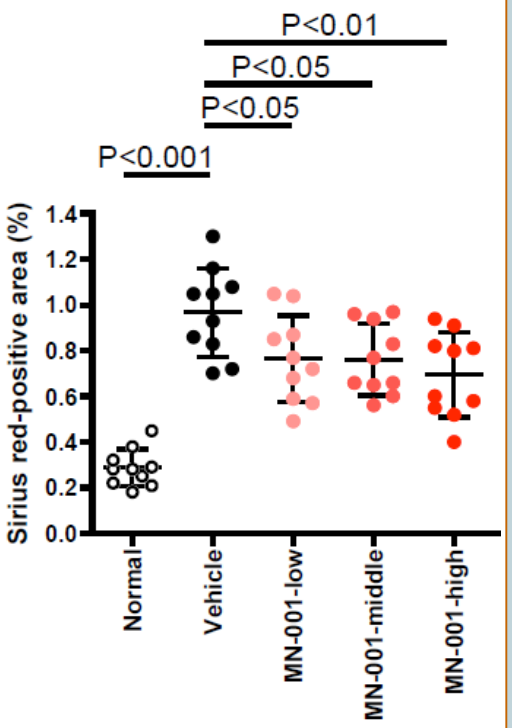
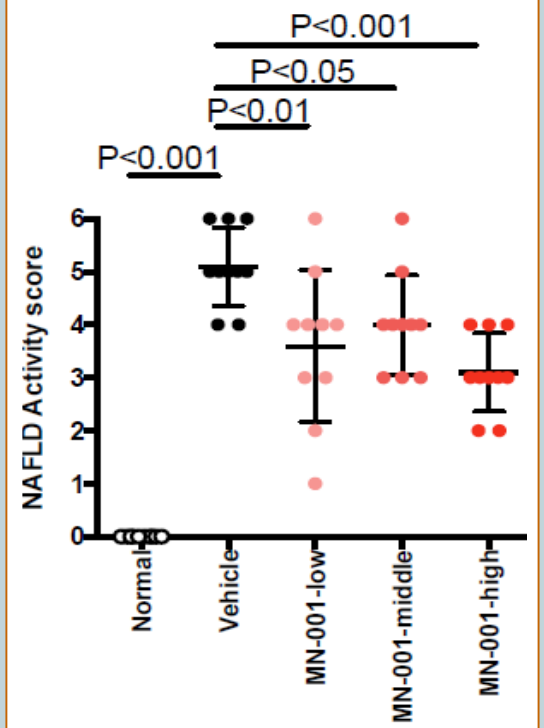
MN-001 Data

NASH & NAFLD Animal Model Studies

% of Fibrosis Area



NAFLD Activity Score (NAS)



Animal model studies shows MN-001 significantly reduced fibrosis in a dose-dependent manner

- Improved NAFLD Activity Score (NAS) via a reduction in hepatocyte ballooning
- Reduced fibrosis area in every preclinical model tested (NASH, Advanced NASH)

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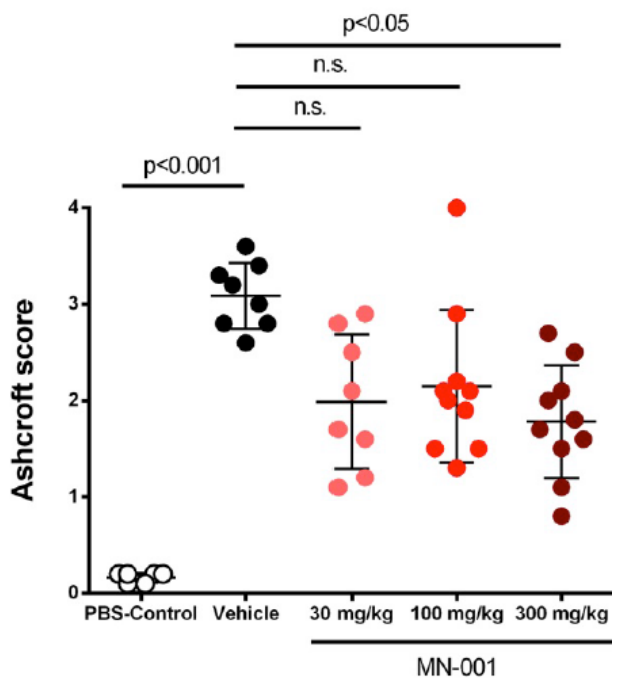
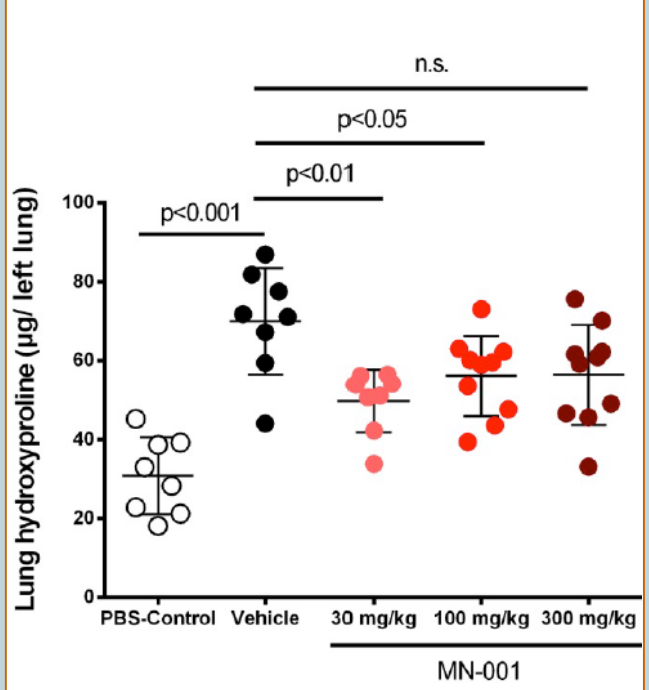
MN-001 Data

IPF Animal Model Study

Ashcroft Score



Lung Hydroxyproline



Animal model study shows MN-001 significantly reduced the Ashcroft Score

- Ashcroft Score measures pulmonary fibrosis based on histopathological staining

MN-001 significantly reduced lung hydroxyproline content

- Hydroxyproline content is an indicator of fibrosis or storage of collagen in tissue

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Nonalcoholic Steatohepatitis (NASH)

Nonalcoholic Fatty Liver Disease (NAFLD)

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Nonalcoholic Steatohepatitis (NASH) and Nonalcoholic Fatty Liver Disease (NAFLD)

NASH AFFECTS

3-12%

of adults in the U.S[1]

PREVALENCE

30-40%

of adults in U.S.[1] have

NAFLD

OVERWEIGHT OR OBESE

NASH MARKET FORECAST

$1.6B

By 2020[2]

NO TREATMENT APPROVED

1. *National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK)*
2. *Allied Market Research*

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MN-001 Phase 2 NASH / NAFLD Trial

Completed

NASH / NAFLD Trial

Subjects with NASH or NAFLD with hypertriglyceridemia

Phase 2 multicenter, proof-of-principle, open-label study

Dosing: MN-001 250 mg once daily for 4 weeks, then twice daily for 8 weeks

INTERIM RESULTS: TRIGLYCERIDES

MN-001 significantly reduced serum triglycerides (primary endpoint)

Mean Serum Triglycerides, baseline	Mean Serum Triglycerides, 8 weeks	Reduction	p-value
328.6 mg/dL (n=15)	192.9 mg/dL (n=15)	41.3%	0.02
260.1 mg/dL (n=14)*	185.2 mg/dL (n=14)*	28.8%*	0.00006

- No clinically significant safety or tolerability issues

STATUS

- Study was terminated early due to positive interim results
- Interim data presented at EASL conference

** Excludes one outlier with extremely high baseline triglyceride level of 1288 mg/dL (300 mg/dL at 8 weeks)*

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Idiopathic Pulmonary Fibrosis (IPF)

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Idiopathic Pulmonary Fibrosis (IPF)

IPF PREVALENCE

132,000 -200,000

in United States[1]

ORPHAN INDICATION

LIFE EXPECTANCY

2-3 YRS[1]

FATAL

IPF MARKET FORECAST

$3B+

By 2025[2]

APPROVED DRUGS

ESBRIET

(pirfenidone)

- Approved in October 2014
- Phase 3 studies enrolled mild to moderate IPF

No survival benefit shown[3]

OFEV

(nintedanib)

- Approved in October 2014
- Phase 3 studies enrolled mild to moderate IPF

No survival benefit shown[4]

1. *Pulmonary Fibrosis Foundation*
2. *GlobalData*
3. *Esbriet prescribing information*
4. *OFEV prescribing information*

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MN-001

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MN-001 Phase 2 IPF Trial

Ongoing

IPF Trial Design	
TRIAL DESIGN	N = 15 subjects with moderate to severe IPF
	Phase 2 randomized, placebo-controlled, double-blind trial at Penn State Milton S. Hershey Medical Center Principal Investigator: Dr. Rebecca Bascom
	Duration: 26 weeks of double-blind treatment + open label extension (26 weeks)
	Dosing: 1500 mg/day of MN-001 or placebo (2:1 randomization)
OBJECTIVES	1) Change from baseline of forced vital capacity (FVC) and FVC % predicted up to 26 weeks, and 2) Semiannual rate of decline of disease activity based on FVC Others: Safety and tolerability; 6-minute walk test (6MWT); Modified Medical Research Council Dyspnea Score (MMRC); quality of life (ATAQ-IPF); frequency of worsening IPF; time to first worsening IPF
STATUS	Ongoing

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Financial Summary

CASH POSITION

$62.9 million

(9/30/2019)

2018 OPERATING CASH BURN

$9.1 million

Consolidated Statements Of Operations And Comprehensive Loss		
Year ended December 31,	**2018**	**2017**
Operating Expenses ($)		
Research, development and patents	$ 5,625,814	$ 4,223,746
General and administrative	9,961,012	8,803,347
Total operating expenses	15,586,826	13,027,093
Operating loss	(15,586,826)	(13,027,093)
Other expense, net	(22,894)	(25,601)
Interest income	939,909	145,508
Loss before income taxes	(14,669,811)	(12,907,186)
Income tax benefit (expense)	(5,276)	1,744,050
Net loss applicable to common stockholders	$ (14,675,087)	$ (11,163,136)
Basic and diluted net loss per common share	$ (0.36)	$ (0.32)
Shares used to compute basic and diluted net loss per share	41,124,909	35,137,028

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Timeline Summary

		2016	2017	2018	2019
MN-166 Ibudilast	**Progressive Multiple Sclerosis**	• Fast Track designation • Interim Analysis: Continue Trial	• Q4: Top-line data presented at ECTRIMS	• Q1: Disability data presented at ACTRIMS • Q3: Results published in New England J. Medicine	• Q2: Results of subgroup analysis • Q3: Announced Phase 3 clinical trial plan in progressive MS
	ALS	• New Patent covers ALS • Initiated ALS Biomarker Study • Interim Data Presented at AAN • Orphan Drug Designation - U.S. • Orphan Medicinal Product Designation - Europe	• Q4: Top-line data presented at International Symposium on ALS/MND	• Q2: Presentation at AAN • Q3: FDA feedback on pivotal trial design	• New patent covers combination of MN-166 (ibudilast) and riluzole • Q2: Announced Phase 3 clinical trial plan in ALS • Q2: Kick-off meeting for Phase 3 trial
	Substance Dependence	• Final Results: Opioid	• Methamphetamine trial initiated at Oregon Health & Science Univ.	• UCLA methamphetamine results reported in Q1 • Two Alcohol trials started	
	DCM			• Q3: announced DCM trial with Univ. of Cambridge	• Q2: Kick-off meeting for DCM trial
	CIPN			• Q1: Initiated CIPN trial	
	Glioblastoma		• Q2: Preclinical data presented at ASCO	• Q2: Opened IND • Q4: Orphan Drug granted	• Q1: Initiated enrollment in glioblastoma clinical trial
MN-001 Tipelukast	**NASH / NAFLD**	• New Patent covers Advanced NASH • Lipid Disorders: New Patent covers hypertriglyceridemia, hypercholesterolemia, and hyperlipoproteinemia		• Q2: Interim data presented at EASL • Terminated study early after positive interim data	• Q1: Research collaboration with Jikei Univ. School of Medicine • Q3: Research collaboration with National Cerebral and Cardiovascular Disease Research Center in Japan
	IPF				• New patents granted in Japan and China

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Investment Highlights

Novel product candidates in clinical development with encouraging efficacy and safety data	
MN-166 (ibudilast)	Treatment of Neurological Diseases i.e. Progressive MS, ALS, Cervical Myelopathy, Peripheral Neuropathy, Glioblastoma, and Substance Dependence • Approved in Japan in 1989 - post-stroke dizziness and asthma • Large safety database
MN-001 (tipelukast)	Treatment of Fibrotic Diseases i.e. IPF (idiopathic pulmonary fibrosis) Treatment of Hyperlipidemia and Fibrotic Disease i.e. NASH (nonalcoholic steatohepatitis) and NAFLD (nonalcoholic fatty liver disease)
Well capitalized	
Experienced management team	

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Appendix

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MN-166

Ibudilast

MN-166 (ibudilast): Patents

Patents that cover MN-166 (ibudilast):	
Progressive Multiple Sclerosis (U.S. Patent 8,138,201)	• Method of treating PPMS or SPMS with ibudilast • Expires no earlier than November 26, 2029 • Foreign patents based on the U.S. patent have been granted in Canada, China, Japan, European Patent Office, Switzerland, Germany, Denmark, Spain, France, United Kingdom, Hungary, Italy, Netherlands, and Sweden
Progressive Multiple Sclerosis (U.S. Patent 8,338,453)	• Method of lessening a conversion of a brain lesion to a persistent black hole in progressive MS using ibudilast • Expires no earlier than July 8, 2028
Progressive Multiple Sclerosis (U.S. Patent 9,114,136)	• Method of reducing brain volume loss in progressive MS using ibudilast • Expires no earlier than July 8, 2028
Amyotrophic Lateral Sclerosis (ALS) (U.S. Patent 9,314,452)	• Method of treating amyotrophic lateral sclerosis (ALS) with ibudilast • Expires no earlier than January 23, 2029
ALS and Neurodegenerative Diseases (U.S. Patent 10,258,611)	• Method of treating ALS and neurodegenerative diseases with ibudilast + riluzole • Expires no earlier than November 24, 2035
Drug Addiction (U.S. Patent 7,915,285)	• Method of treating drug addiction or drug dependence with ibudilast • Expires no earlier than January 27, 2030 • Foreign patents based on the U.S. patent have been granted in Japan, European Patent Office, Germany, Spain, France, United Kingdom, and Italy
Neuropathic Pain (U.S. Patent 7,534,806)	• Method of treating neuropathic pain with ibudilast • Expires no earlier than December 6, 2025

Note: Expiration dates listed above do not include patent term restoration which would add up to 5 years extension.

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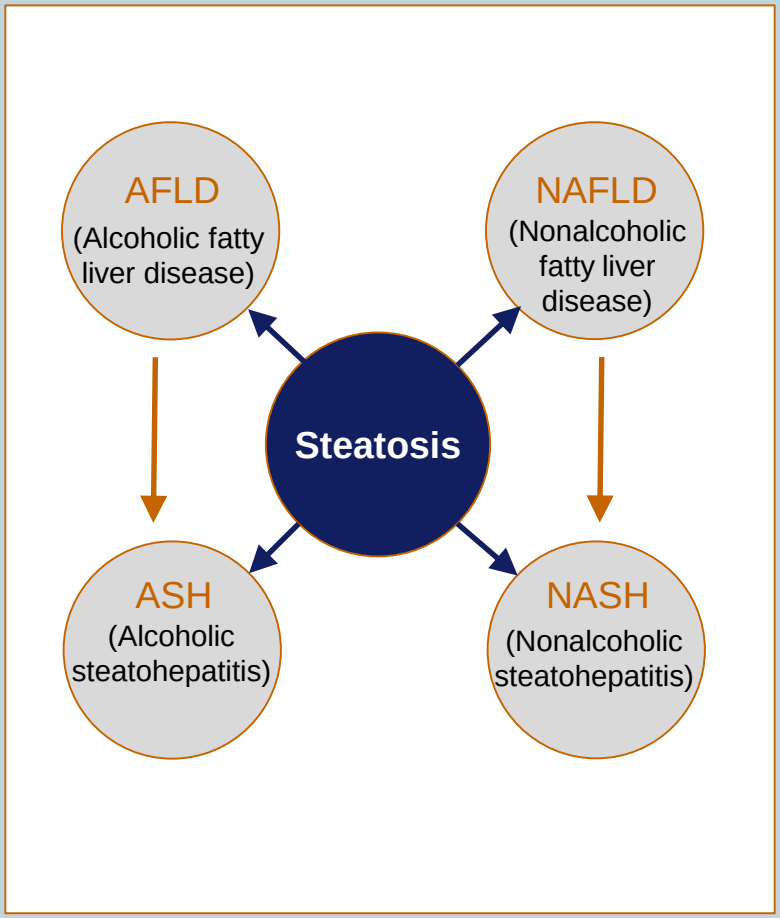
MN-001
Tipelukast

MN-001 (tipelukast): 6 New Patents

6 New Patents cover MN-001 (tipelukast) and MN-002 (a major metabolite of MN-001):	
NASH	Treatment of nonalcoholic steatohepatitis (NASH); expires no earlier than Dec 2032
Advanced NASH	Treatment of advanced NASH with fibrosis; expires no earlier than Sep 2034
NAFLD	Treatment of nonalcoholic fatty liver disease (NAFLD); expires no earlier than Dec 2032
Liver Disorders	Treatment of steatosis, lobular inflammation, hepatic ballooning, hepatic scarring, and elevated liver hydroxyproline levels; expires no earlier than Dec 2032
Lipid Disorders	Treatment of hypertriglyceridemia, hypercholesterolemia, and hyperlipoproteinemia; expires no earlier than July 2034
Fibrosis	Treatment of fibrosis in a broad range of organs; expires no earlier than June 2035



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