



CYTOKINETICS

**CYTOKINETICS ANNUAL
STOCKHOLDER MEETING
*MAY 21, 2014***

Forward-Looking Statements

Statements in and/or made by our representatives in connection with this presentation regarding future events or our future performance are forward-looking statements. We intend that such statements be protected by the Private Securities Litigation Reform Act of 1995's safe harbor. Our actual results and the timing of events may differ materially from those projected in these statements. Examples of such statements include, but are not limited to, statements relating to: our and our partners' research and development programs, including plans for and the initiation, design, conduct and results of clinical trials, the significance of such results; the anticipated timing of events; the commercial potential for our drug candidates and market potential for our targeted indications; our financial guidance and R&D milestones; our receipt of funds and anticipated role in development and commercialization activities under our agreements with Amgen and Astellas; the properties and potential benefits of our compounds, including their potential indications; and the utility of our focus on muscle function and contractility.

These forward-looking statements involve many risks and uncertainties that could cause actual results and the timing of events to differ materially from those projected by these statements. These risks and uncertainties include a variety of factors, many of which are beyond our control. These statements speak as of today, and you should not rely on them as representing our views in the future. We undertake no obligation to update these statements after this presentation. Please refer to our SEC filings, including our annual reports filed on Form 10-K, our periodic reports filed on Form 10-Q and our current reports filed on Form 8-K, for a more detailed description of these risks and uncertainties. Copies of these documents may be obtained from the SEC or by visiting the Investor Relations section of our website.



Great ideas
originate in
the muscles.

Thomas A. Edison



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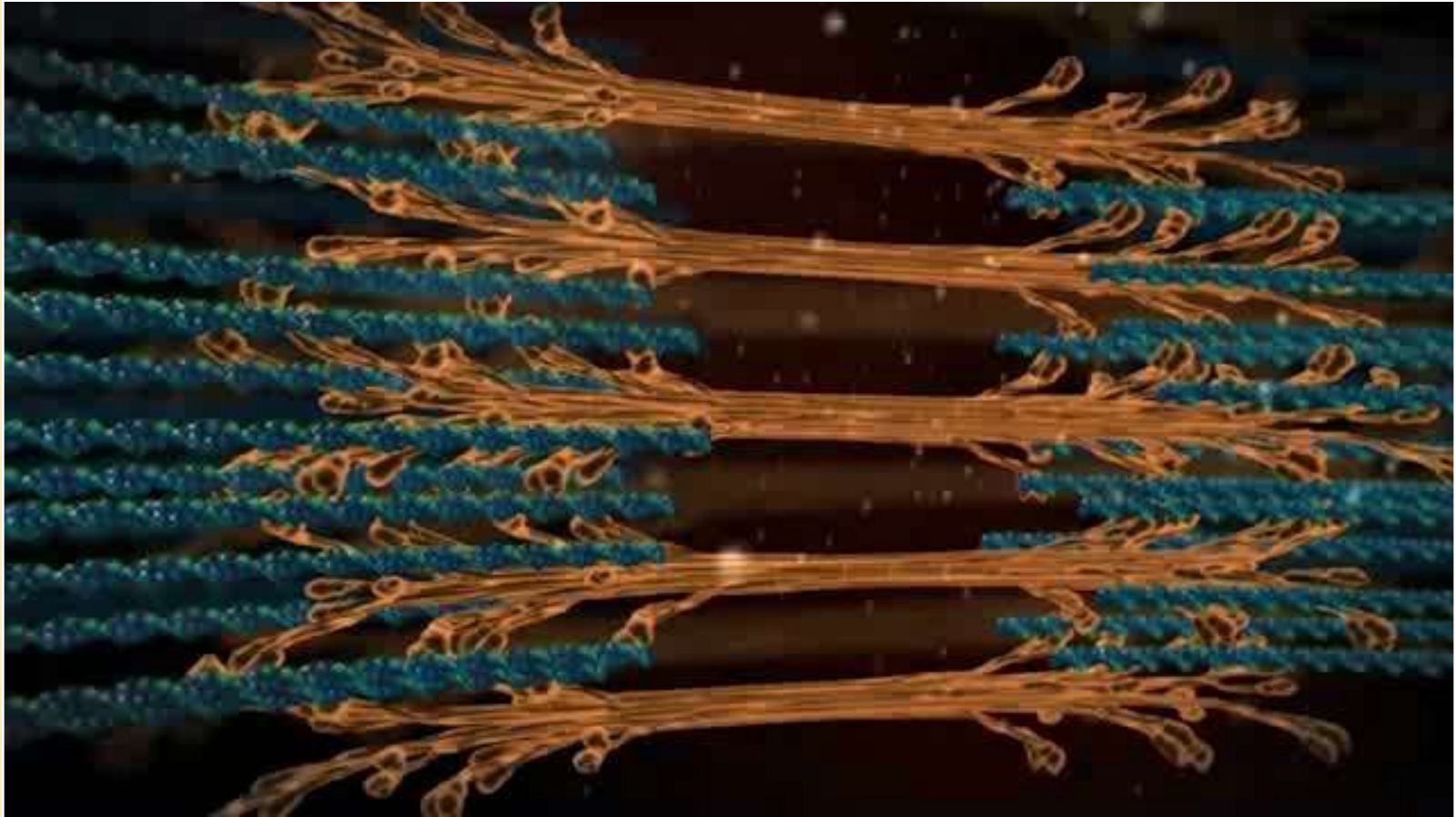


Progress in 2013 Reinforces Outlook for 2014

- **Advancing multiple muscle biology directed programs**
- **Achieved clinically relevant effects in Phase IIb trials**
- **Transacted corporate development deals to diversify risk**
- **Positioned well for continued progress in late stage development**
- **Strong balance sheet and diversified funding sources**
- **Continued commitment to patients with severe diseases**



Sarcomere: The Fundamental Unit of Muscle Contractility



Link to video: <http://www.cytokinetics.com/video>

Cytokinetics' Development Pipeline in 2014

	IND	Phase I	Phase IIa	Phase IIb
Skeletal Muscle				
<i>tirasemtiv (neuromuscular)</i>				
CK-2127107 (non-neuromuscular)		Astellas Collaboration		
Cardiac Muscle				
<i>omecamtiv mecarbil – iv (heart failure)</i>		Amgen Collaboration		
<i>omecamtiv mecarbil – oral (heart failure)</i>		Amgen Collaboration		

**First-in-class Programs in Later-Stage Development
That Leverage Extensive Phase I And Phase IIa Results**



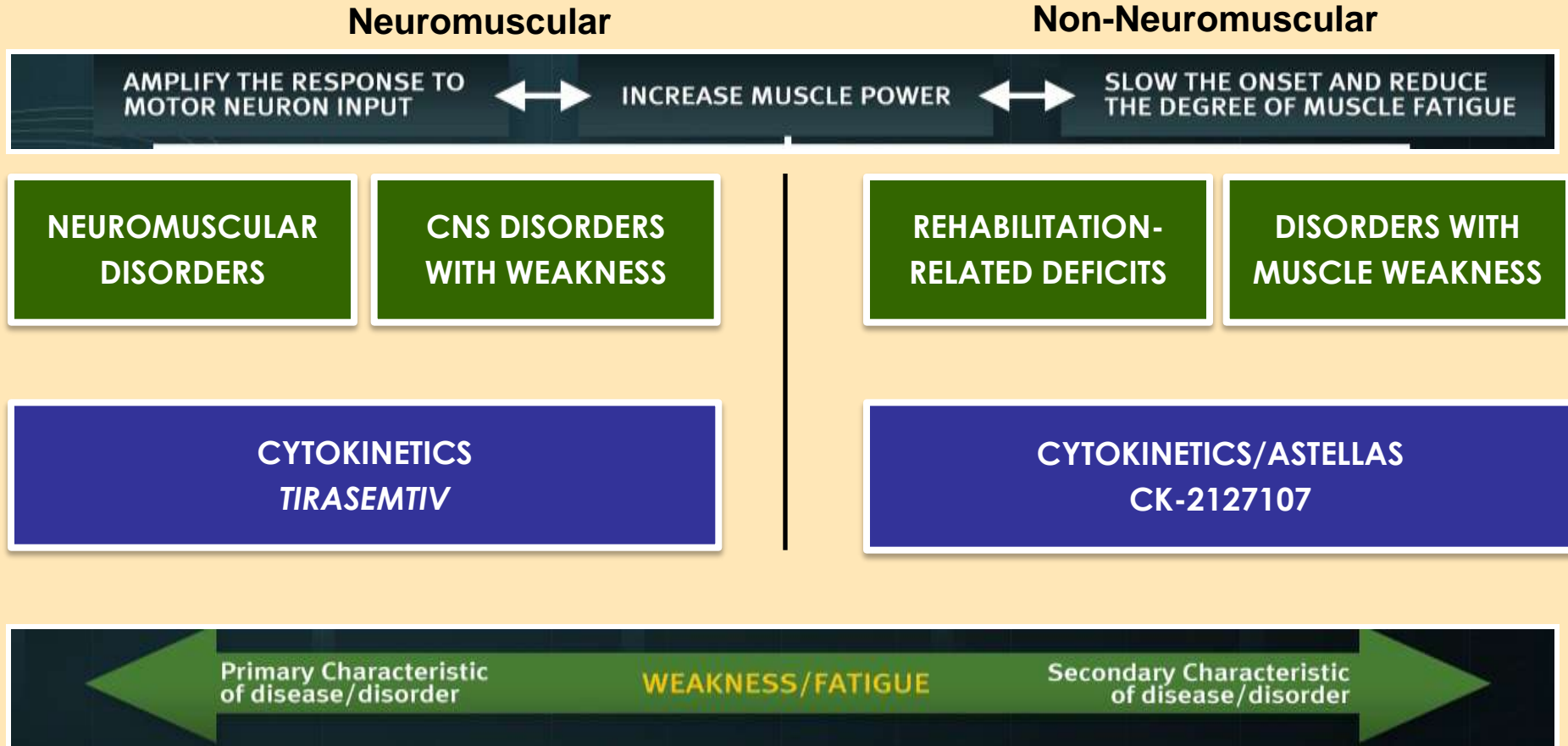
Investigational products – not approved as safe or effective for any indication.

SKELETAL MUSCLE PROGRAM

TIRASEMTIV
CK-2127107



Skeletal Muscle Program: Breadth of Opportunities



Multiple Opportunities for Fast Skeletal Troponin Activators



TIRASEMTIV



Tirasemtiv: Phase I and II Clinical Trials

POPULATION (STUDY #)	N	FORM	TRIAL OBJECTIVE	RESULTS	STATUS
Phase I					
Healthy Subjects (CY 4011 Part A)	57	Oral	Assess safety and tolerability; Evaluate pharmacokinetics (increasing single doses)	MTD determined to be 2000 mg No Serious Adverse Events; Well tolerated	Announced Feb 2010
Healthy Subjects (CY 4011 Part B)	12	Oral	Asses pharmacodynamic effects	Concentration-dependent, statistically significant increases (versus placebo) in peak force; Well tolerated	Announced Jan 2010
Healthy Subjects (CY 4012)	24	Oral	Assess safety and tolerability; Evaluate pharmacokinetics (once-daily for 7 days)	Dose proportional Cmax & AUC24h; low inter-subject variability Modest accumulation from single-dose to stead state No Serious Adverse Events; Well tolerated	Announced Jan 2010
Healthy Subjects (CY 4013)	36	Oral	DDI (<i>riluzole</i>) and Food Effect Study; Safety and tolerability	Standard adjustment to <i>riluzole</i> dose to be made Best administered to patients in a fasting state	Announced Oct 2011
Phase II					
ALS Patients (CY 4021)	67	Oral	Hypothesis generating; Safety and tolerability; Assess PK/PD effects	Positive changes in patients' overall status at 6 hours Improvements in SNIP, MVV and Grip Strength Endurance Well tolerated as a single dose	Announced Dec 2010
ALS Patients (CY 4024 Part A)	24	Oral	Safety and tolerability in multiple fixed doses (14 days duration without <i>riluzole</i>)	Well tolerated over 2 weeks of fixed QD dosing w/o <i>riluzole</i> Dose-related trends to improvement in ALSFRS-R scale and MVV	Announced Nov 2011
ALS Patients (CY 4024 Part B)	25	Oral	Safety and tolerability in multiple fixed doses (14 days duration with <i>riluzole</i>)	Well tolerated over 2 weeks of fixed QD dosing w/ <i>riluzole</i> Dose-related trends to improvement in ALSFRS-R scale and MVV	Announced April 2012
ALS Patients (CY 4025)	27	Oral	Safety and tolerability in multiple ascending doses (14 days duration with <i>riluzole</i>)	Well tolerated for 3 weeks BID dose escalation w/ <i>riluzole</i> Dose-related trends to improvement in ALSFRS-R scale and MVV	Announced April 2012
ALS Patients (BENEFIT-ALS)	711	Oral	Assess ALSFRS-R and secondary measures of skeletal muscle function	Did not improve ALSFRS-R; Statistically significant increase in SVC and Muscle Mega-Score	Announced April 2014
Claudication Patients (CY 4022)	61	Oral	Hypothesis generating; Safety and tolerability; Assess PK/PD effects	Increased calf muscle performance by heel raise testing Well tolerated	Announced June 2011
Myasthenia Gravis (CY 4023)	32	Oral	Hypothesis generating; Safety and tolerability; Assess PK/PD effects	Improvements in QMG and FVC at 6 hours after dosing	Announced Nov 2012

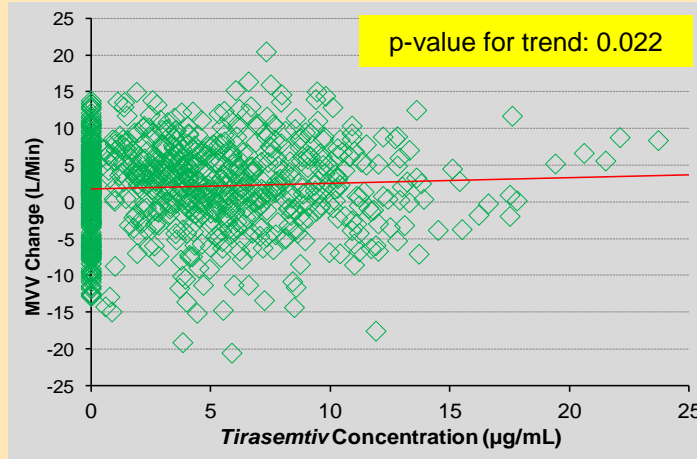
**Well Characterized Safety, Tolerability, PK/PD
In Healthy Volunteers and ALS Patients**



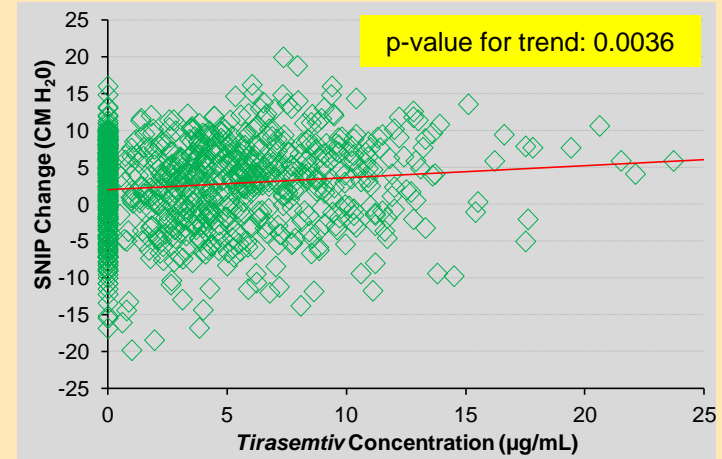
Tirasemtiv: Effects Increase with Plasma Concentration*

Meta-Analysis of Three Previously Completed Phase IIa Clinical Trials

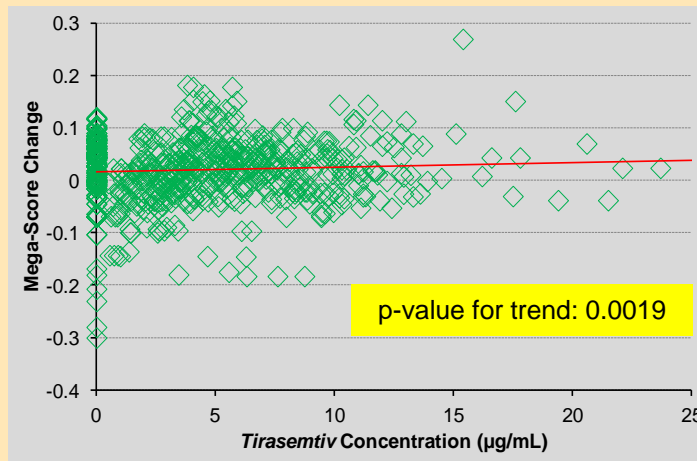
Maximal Voluntary Ventilation (MVV, L/min)



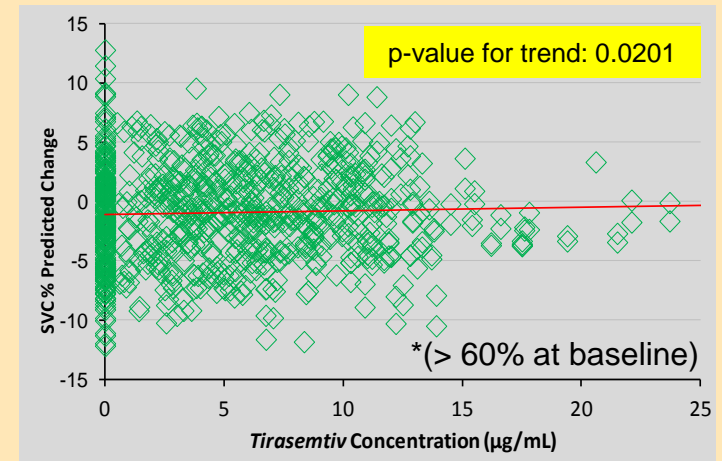
Sniff Nasal Inspiratory Pressure (SNIP, cm H₂O)



Muscle Strength Mega-Score



Slow Vital Capacity* (SVC, % Predicted)

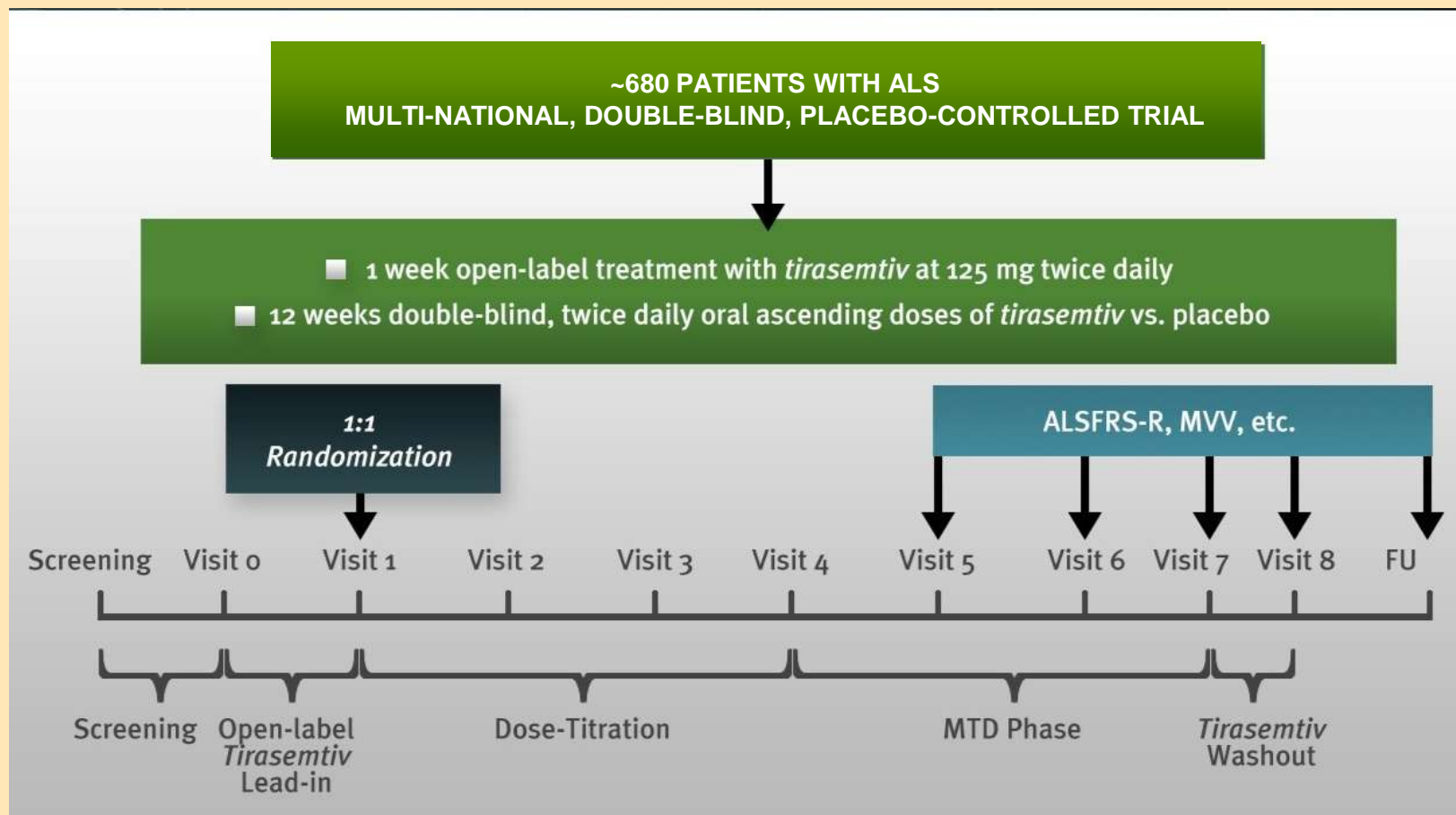


*Placebo-corrected Changes from Baseline

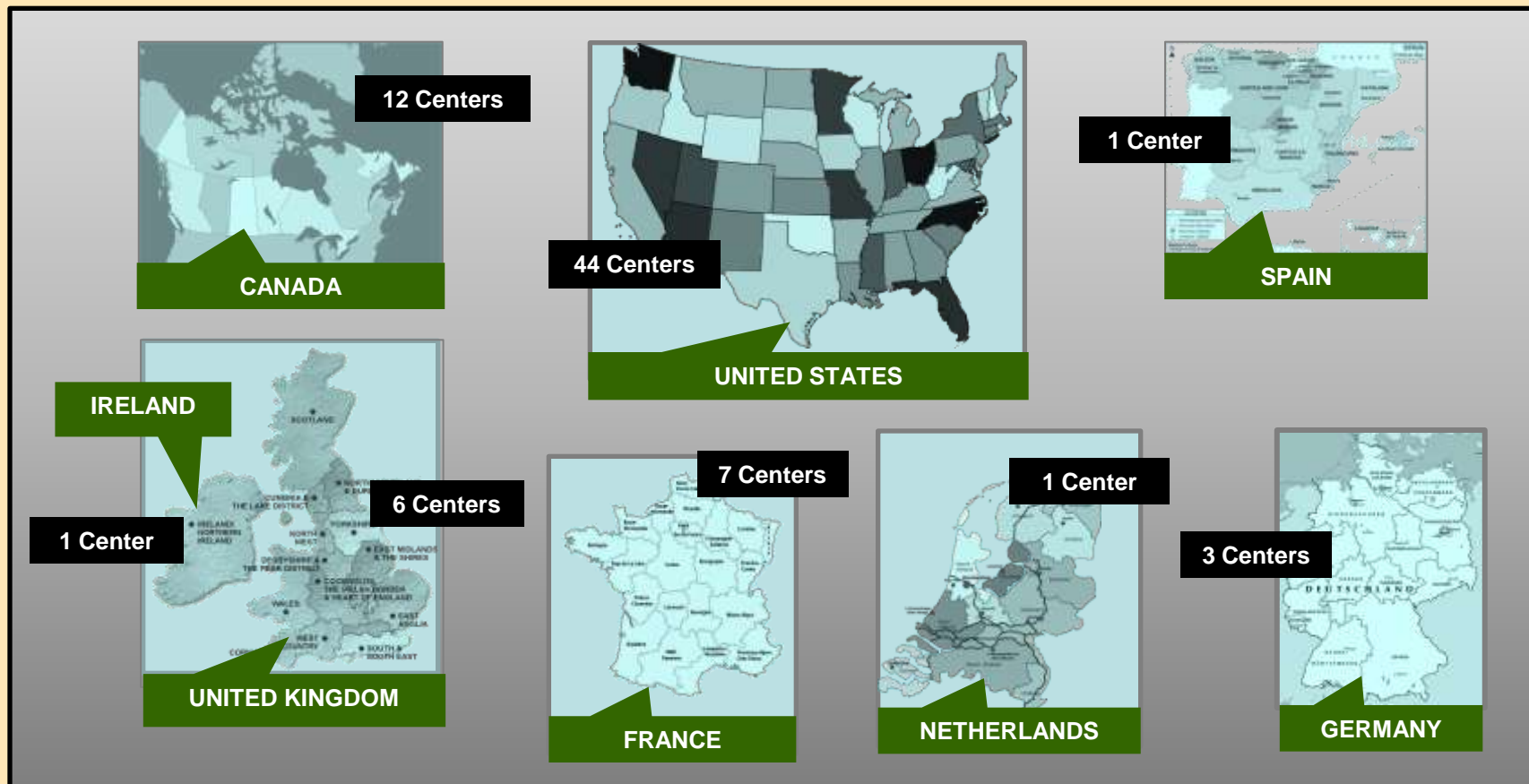
Shefner JM, et al. *Amyotroph Lateral Scler Frontotemporal Degener*, 2013 Dec;14(7-8):582-5.

BENEFIT-ALS: Phase IIb Clinical Trial

Blinded Evaluation of Neuromuscular Effects and Functional Improvement with *Tirasemtiv* in ALS



BENEFIT-ALS: Enrollment by Country



711 Patients Enrolled

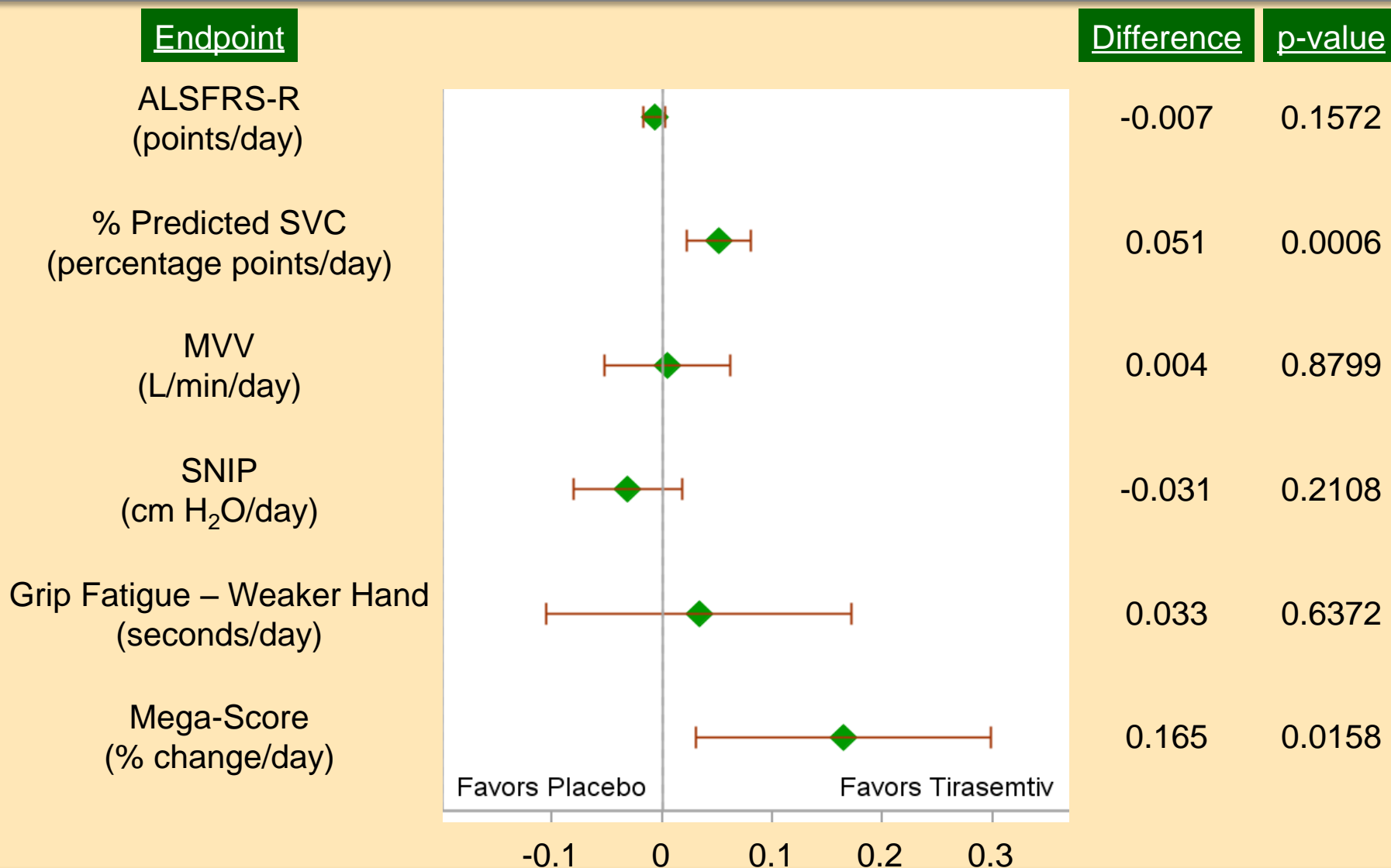


BENEFIT-ALS: Primary Efficacy Endpoint: ALSFRS-R

	Placebo (n = 210)	<i>Tirasemtiv</i> (n = 178)	All (N = 388)
Baseline (Mean \pm SD)	37.3 (4.2)	37.0 (4.7)	37.2 (4.4)
Changes from Baseline (LSM \pm SE) [n]			p-value
Visit 6 (Week 8)	-2.00 (0.23) [204]	-2.49 (0.26) [151]	0.160
Visit 7 (Week 12)	-2.81 (0.28) [199]	-3.48 (0.31) [144]	0.107
Average of Visits 6 and 7	-2.40 (0.25) [204]	-2.98 (0.28) [151]	0.114



BENEFIT-ALS: Slope Difference for *Tirasemtiv* vs. Placebo



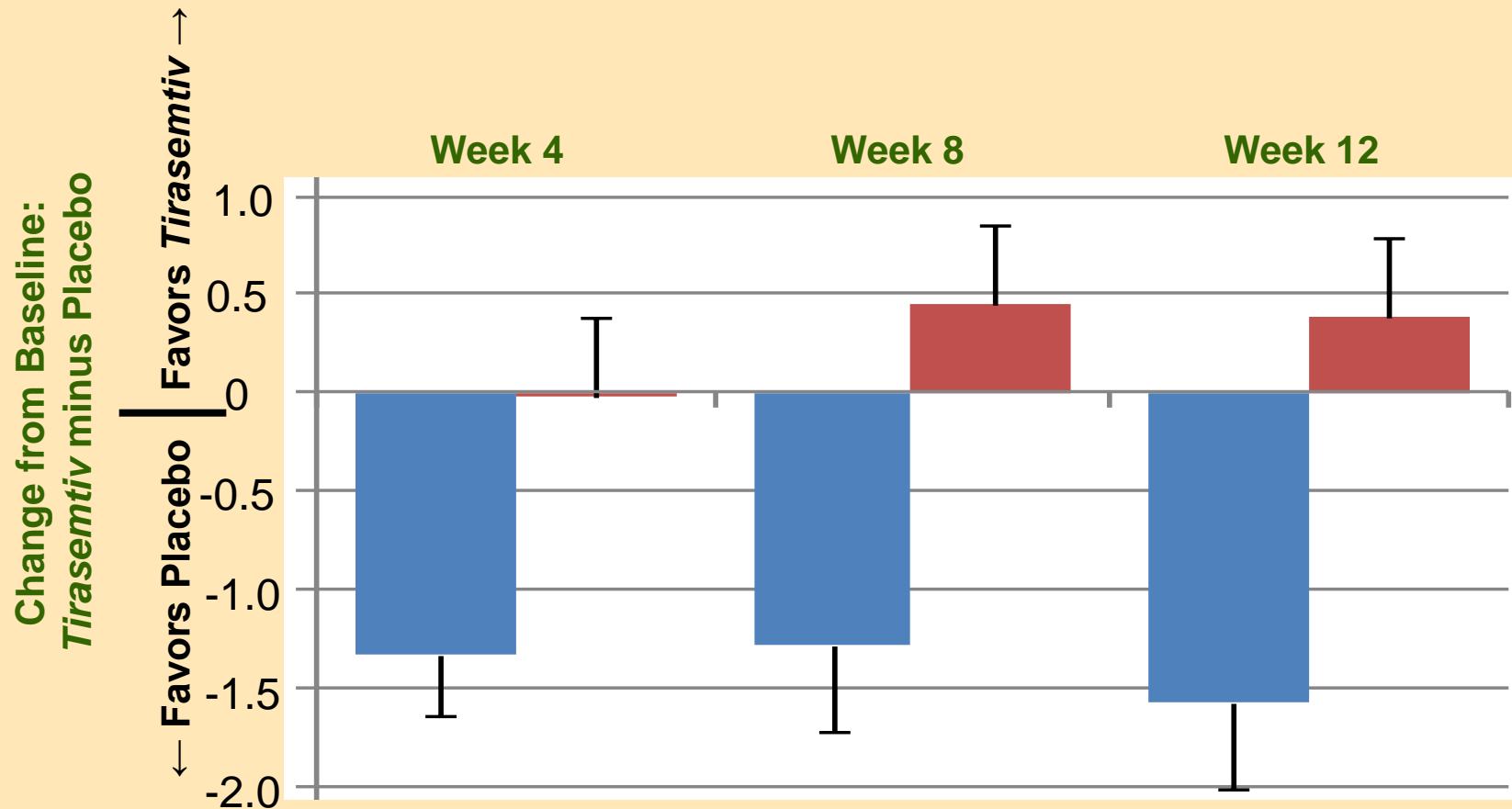
BENEFIT-ALS: Slow Vital Capacity

	Placebo (n = 210)	<i>Tirasemtiv</i> (n = 178)	All (N = 388)
Baseline (% Predicted, mean \pm SD)	89.7 (17.2)	85.7 (19.3)	87.8 (18.3)
Changes from Baseline (LSM \pm SE)			p-value
Week 4 (pre-dose)	-3.89 (0.62)	-0.99 (0.68)	0.001
Week 8 (pre-dose)	-5.81 (0.68)	-2.85 (0.77)	0.004
Week 12 (pre-dose)	-8.66 (0.80)	-3.12 (0.90)	<0.0001



BENEFIT-ALS: Weight Loss - Negative Impact on ALSFRS-R

ALSFRS-R Changes from Baseline: *Tirasemtiv* versus Placebo



Weight Change-by-Treatment Interaction $p = 0.052$



Weight change on *Tirasemtiv* ≤ Median (-1.36 Kg, n = 90)
Weight change on *Tirasemtiv* > Median (-1.36 Kg, n = 86)

Tirasemtiv (n = 176) :
Placebo (n = 210)



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Tirasemtiv: Progression to Potential Registration

2010 - 2012

CY 4021

Phase IIa (Evidence of Effect)

A Phase IIa EoE, Double-blind, Randomized, Placebo-Controlled, Three-Period Crossover, PK/PD Study to Evaluate *tirasemtiv* in Male and Female Patients With ALS.

CY 4024

Phase II

Part A (w/o *riluzole*) Part B (w/ *riluzole*)

A Phase II, Double-Blind, Randomized, Placebo-Controlled Study to Evaluate the Effects of Multiple Doses of *tirasemtiv* in Patients With ALS.

CY 4025

Phase II

A Phase II, Multicenter, Double-Blind, Randomized, Placebo-Controlled Dose Titration Study to Evaluate the Safety, Tolerability and Pharmacodynamic Effects of *tirasemtiv* in Patients With ALS.

2013 – 2014

BENEFIT-ALS

FDA



EMA

**Data
Q2 2014**

Reviewing
Next
Steps

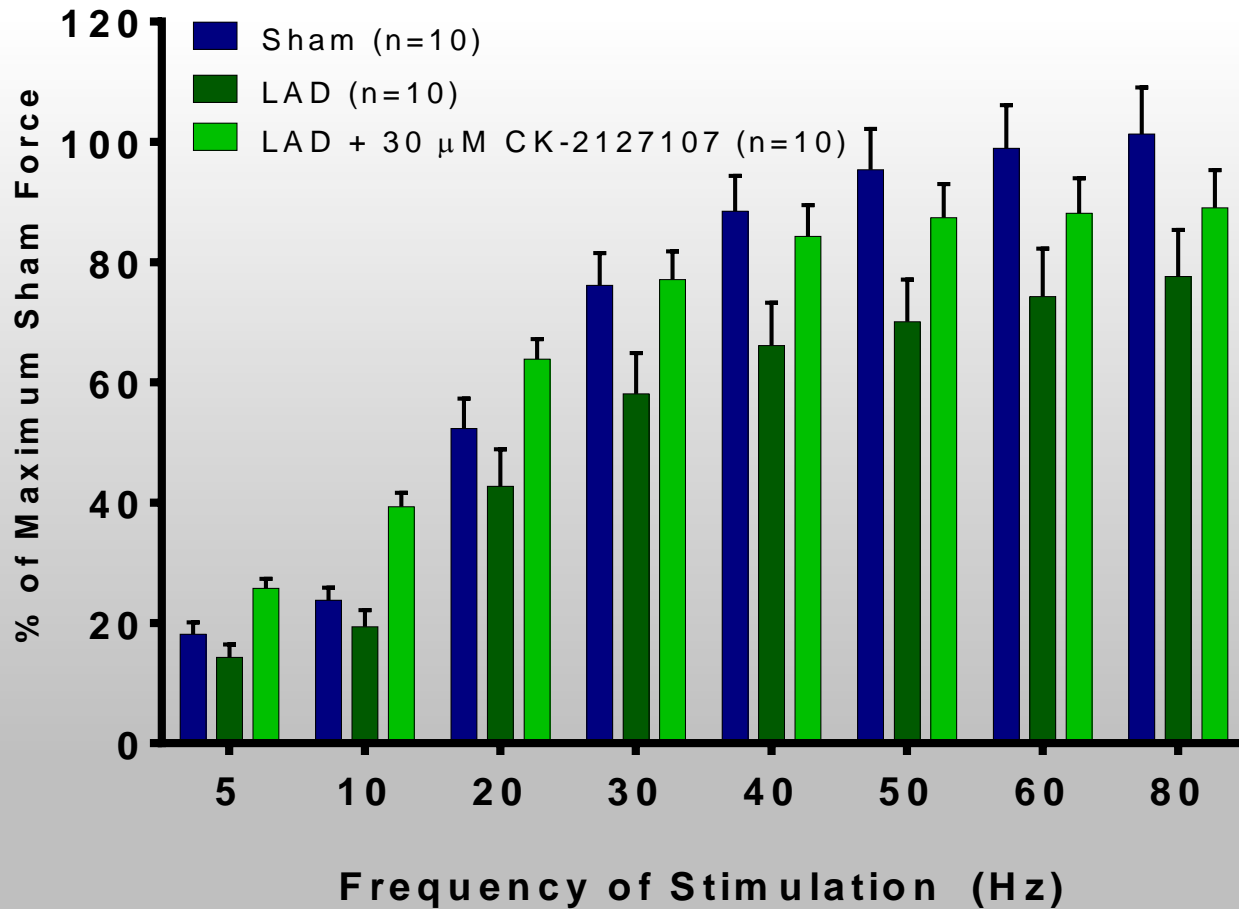


CK-2017107

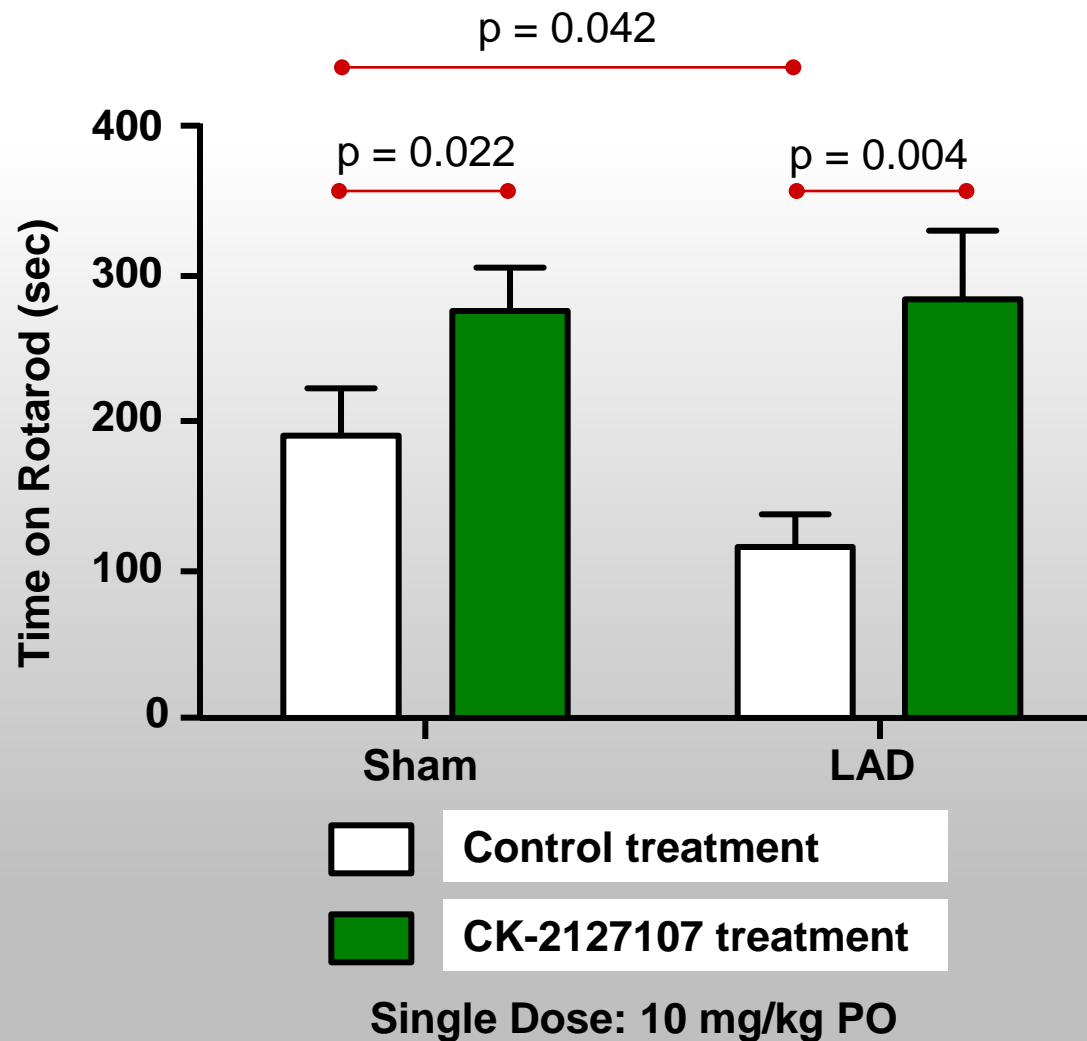


CK-2017107: Restores Function in Muscle Fibers

Living Diaphragm Muscle
Force – Frequency relationship



CK-2127107: Improves Running Performance in Rat Model



CK-2127107: First-Time-In-Humans Clinical Trial

- Well tolerated up to 4000 mg
- No emerging pattern of adverse events
- Maximum tolerated dose was not reached
- Adequate exposures achievable via oral dosing
- Linear, dose proportional PK observed up to 4000 mg
- Terminal $t_{1/2}$ compatible with once or twice daily dosing

**Additional Phase I Clinical Trials in 2014
to Support Potential Progression to Phase II**



CARDIAC MUSCLE PROGRAM

OMECAMTIV MECARBIL



Omecamtiv Mecarbil: Phase I Clinical Trials

Study #	N	Form	Trial Objectives	Results	Status
Phase I					
Healthy Volunteers* (CY 1111)	34	IV	Safety and Tolerability MTD / Plasma Concentration	PK: Linear, Dose Proportional Echo: Dose and concentration dependent increases in cardiac function Safety: Well- tolerated up to MTD	Announced 2006
Healthy Volunteers (CY 1011)	10	IV Oral	Oral Bioavailability	100% Bioavailability No first-pass hepatic metabolism	Announced 2006
Healthy Volunteers (CY 1016)	12	Oral	Modified Release Pharmacokinetics	Prototype selected	Announced June 2008
Healthy Volunteers (CY 1015)	32	Oral	Single dose to multi-dose Pharmacokinetics	Dose-proportionality No gender differences	Announced June 2008
Healthy Volunteers (CY 1013)	24	Oral	Drug/Drug Interaction	Absence of metabolism by CYPs 3A4 and 2D6 had minimal effect on <i>omecamtiv mecarbil</i> pharmacokinetics	Announced December 2008
Healthy Volunteers (AMG 20090727)	65	Oral	Modified Release Pharmacokinetics	MR formulations selected for study in Ph2	Announced 2012
Renal Patients (AMG 20080676)	12	Oral	Safety and Tolerability Pharmacokinetics	No clinically meaningful differences in <i>omecamtiv mecarbil</i> pharmacokinetics in patients undergoing hemodialysis	Completed 2013
Healthy Volunteers (CY 1211)		Oral	Safety and Tolerability Pharmacokinetics (Japanese vs. Caucasian)	-	Initiating



*Teerlink et al.
Lancet 2011

**Cleland et al.
Lancet 2011

Omecamtiv Mecarbil: Phase II Clinical Trials

Study #	N	Form	Trial Objectives	Results	Status
Stable Heart Failure** (CY 1121)	45	IV	Safety and tolerability, PK/PD dose-response	<u>Safety</u> : Well-tolerated <u>Statistically significant increases</u> : Stroke Volume, Fractional Shortening, Systolic Ejection Time, Ejection Fraction	Announced March 2009
Ischemic Cardiomyopathy (CY 1221)	94	IV to Oral	Safety	<u>Safety</u> : Well-tolerated Supports progression into Phase IIb	Announced June 2009
ATOMIC-AHF	613	IV	Safety and tolerability, PK/PD, potential efficacy	<u>Safety</u> : Overall SAE profile and tolerability similar to placebo <u>PK</u> : Similar to healthy volunteers and stable HF patients <u>PD</u> : Systolic ejection time significantly increased consistent with MOA <u>Efficacy</u> : Statistically significant dose and concentration related improvements in dyspnea but 1 ^o endpoint not met	Announced September 2012
COSMIC-HF	~450	Oral	Safety and tolerability, PK/PD	-	Ongoing

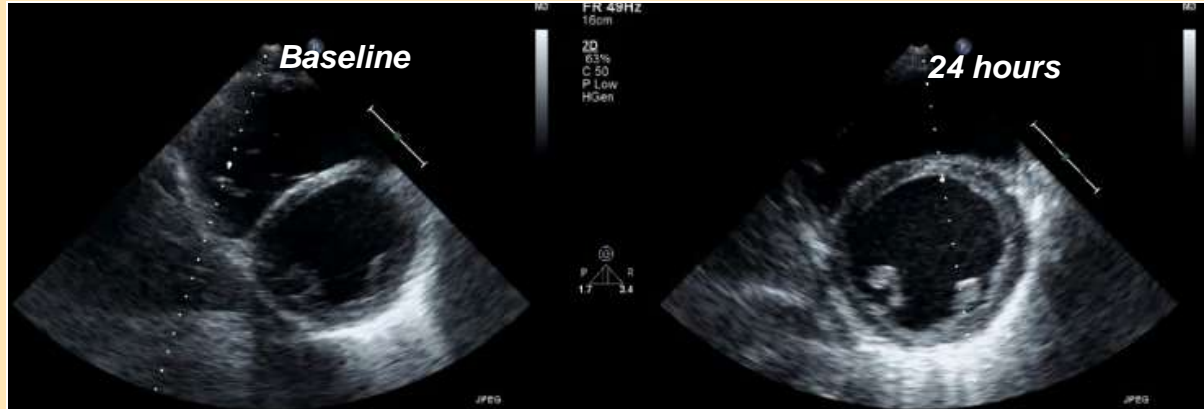


*Teerlink et al.
Lancet 2011

**Cleland et al.
Lancet 2011

Omecamtiv Mecarbil: Echocardiographic Data

Short Axis & 2 Chamber views

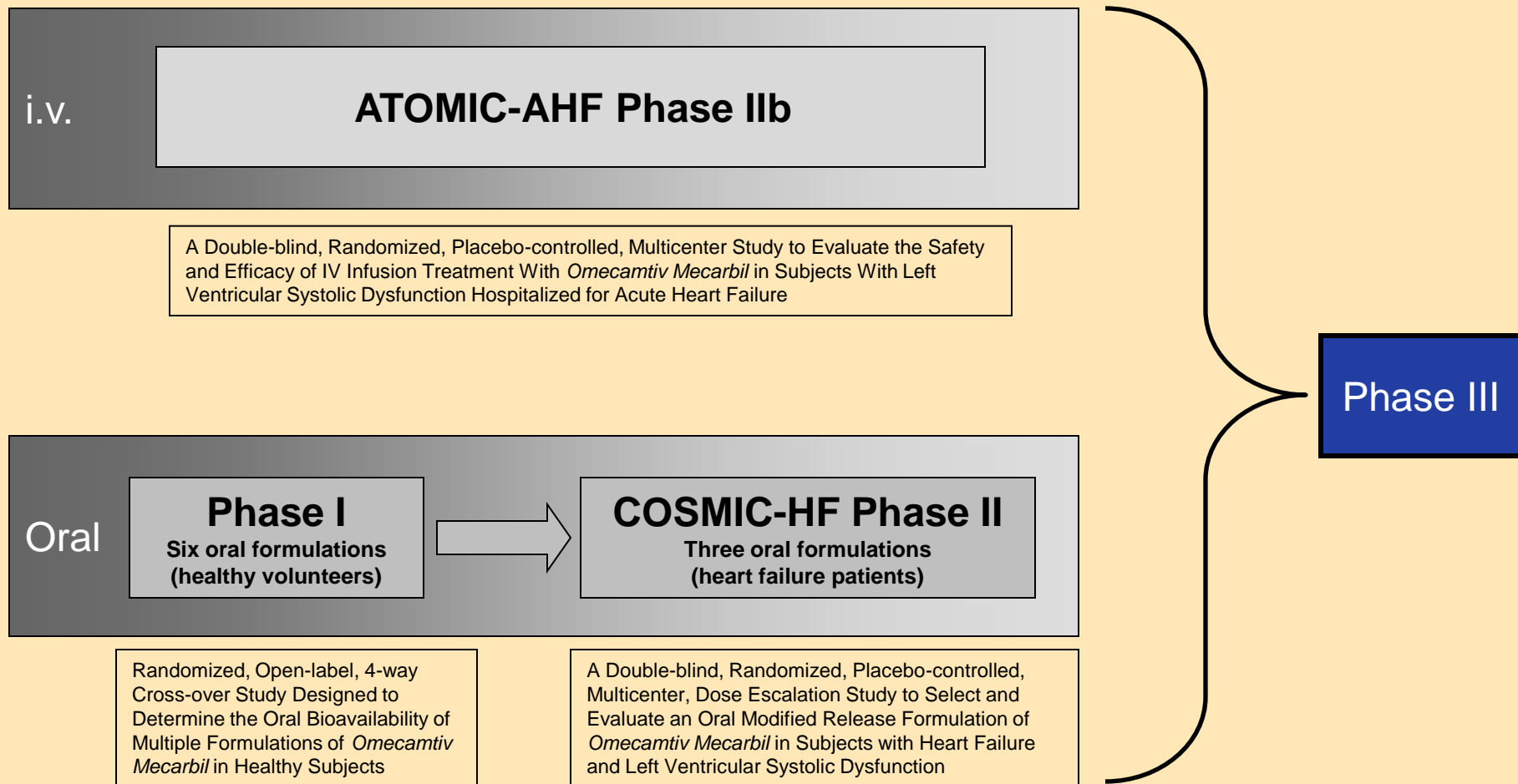


	SET (msec)		LVOT SV (mL)		EF (%)		HR (bpm) – supine ECG	
	Baseline	24 hrs	Baseline	24 hrs	Baseline	24 hrs	Baseline	24 hrs
<i>omecamtiv mecarbil</i>	216	311	23	54	18	23	88	57
Placebo	234	225	26	24	18	18	85	86



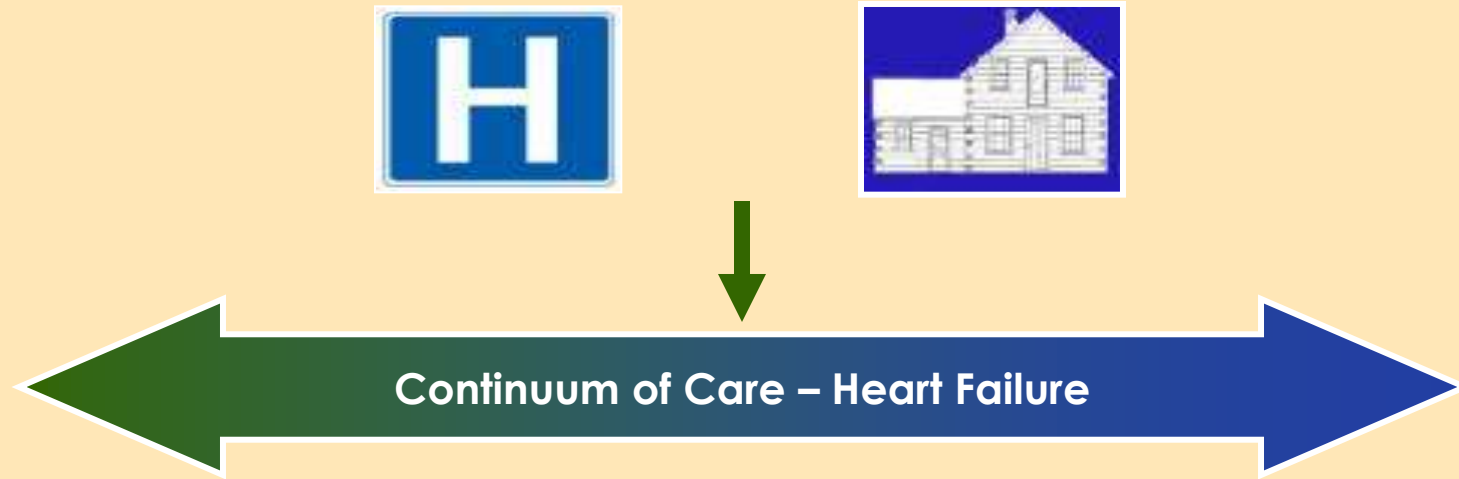
Link to video: http://www.cytokinetics.com/echoslide_2014

Omecamtiv Mecarbil: Progression to Potential Phase III



Omecamtiv Mecarbil: Potential Registration Strategy

Continuum of Care



Potential Indication(s):

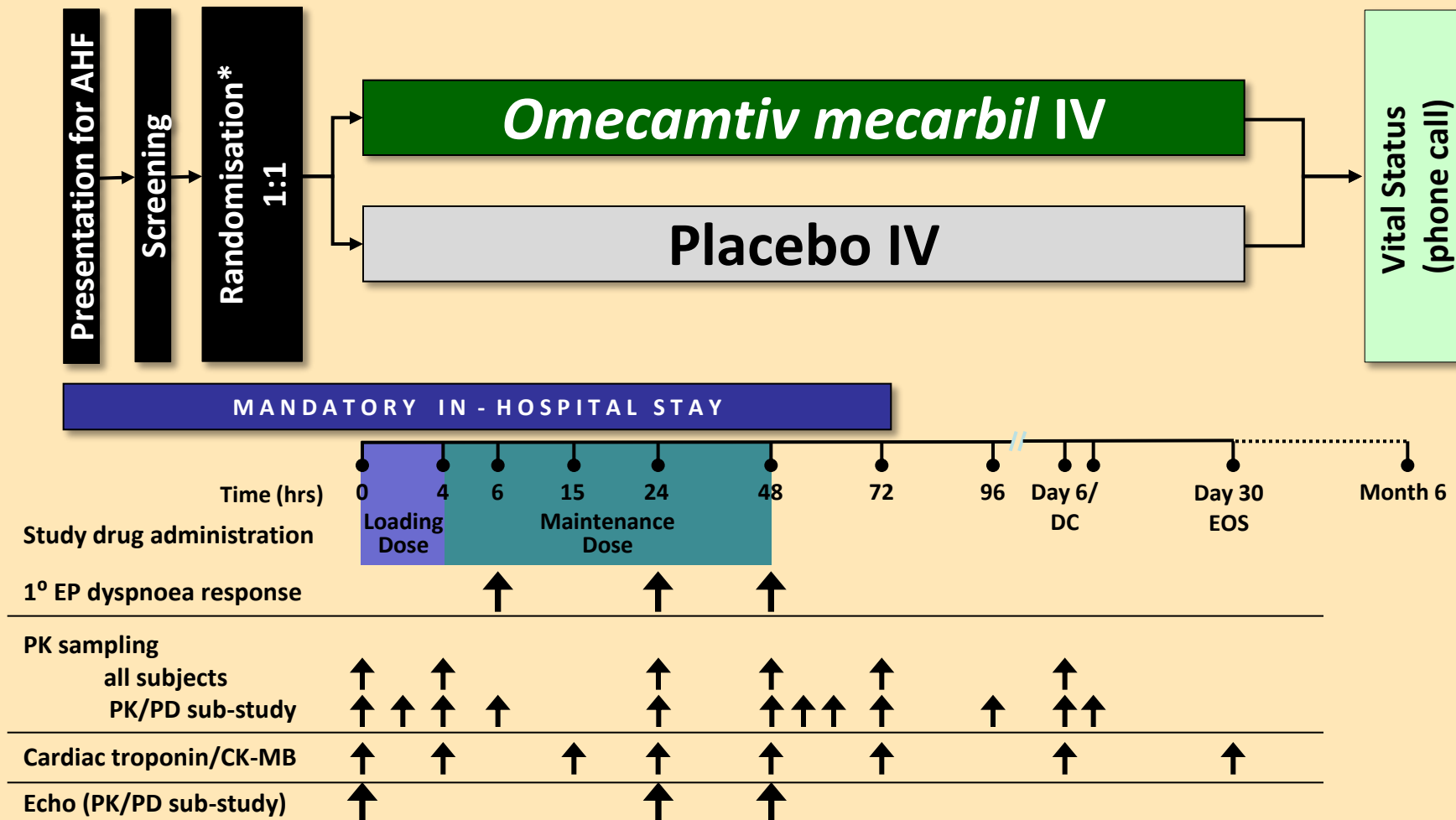
- Reduction in death or readmission
- Improvement in symptoms and functional status

Objective is to Develop Therapeutic Regimen that Addresses Continuum of Care to Reduce Re-Hospitalization and Death



ATOMIC-AHF: Study Design

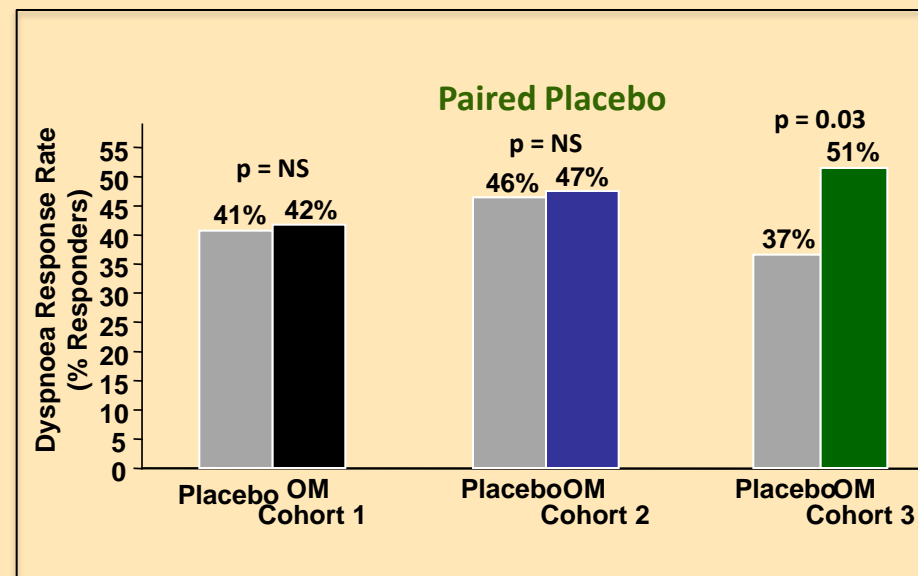
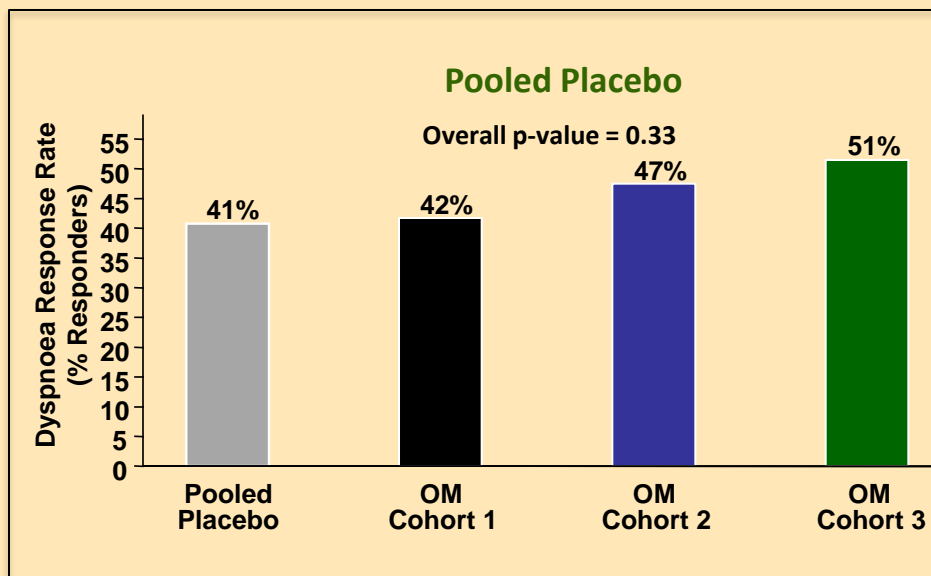
Randomised, double-blind, placebo-controlled, sequential cohort study



* Randomisation within 24 hours of initial IV diuretic (Amendment 2)



ATOMIC-AHF: Dyspnea Response (Likert Scale)



Response Rate Ratio*	1.03	1.15	1.23
95% CI	(0.79, 1.35)	(0.90, 1.47)	(0.97, 1.55)

Response Rate Ratio	1.02	1.02	1.41
95% CI	(0.74, 1.42)	(0.76, 1.37)	(1.02, 1.93)

*Ratio of response rate to Pooled Placebo
p-value of a CMH test among all 3 Placebo arms = 0.32

Response rate ratio:
ratio of response rate to Placebo within each cohort



ATOMIC-AHF: Dose and Concentration Relationship

	For Increases of...	Response Rate Ratio Increases...	95% CI	P-value
Dose*	50 mg total OM administered	5.5%	0.7% – 10.6%	0.025
Plasma concentration*	4000 hr*ng/mL AUC48h	6.4%	1.7% – 11.4%	0.007

* Adjusted for region, cohort, age, baseline NRS, presentation-randomisation duration (continuous)



ATOMIC-AHF: Worsening Heart Failure (WHF)

Within 7 days of IP initiation	Pooled Placebo (N = 303)	Cohort 1 OM (N = 103)	Cohort 2 OM (N = 99)	Cohort 3 OM (N = 101)
Death or WHF*				
Yes - n(%)	52 (17)	13 (13)	9 (9)	9 (9)
Relative risk		0.67	0.54	0.54
(95% CI)		(0.38, 1.18)	(0.28, 1.04)	(0.27, 1.08)
p-value		0.151	0.054	0.067
WHF*				
Yes - n(%)	51 (17)	13 (13)	8 (8)	9 (9)
Relative risk		0.68	0.49	0.55
(95% CI)		(0.38, 1.21)	(0.24, 0.98)	(0.28, 1.09)
p-value		0.179	0.034	0.075

*Worsening heart failure is defined as clinical evidence of persistent or deteriorating heart failure requiring at least one of the following treatments:

- Initiation, reinstitution or intensification of IV vasodilator
- Initiation of IV positive inotropes, or IV vasopressors
- Initiation of ultrafiltration, hemofiltration, or dialysis
- Initiation of mechanical ventilatory or circulatory support



ATOMIC-AHF: Reductions in Tachyarrhythmias

Preferred Term Patient Incidence, n (%)	Pooled Placebo (N = 303)	Pooled OM (N = 303)	Cohort 1 OM (N = 103)	Cohort 2 OM (N = 99)	Cohort 3 OM (N = 101)
Number of subjects reporting AEs of Supraventricular or Ventricular Tachyarrhythmia	34 (11.2)	26 (8.6)	13 (12.6)	5 (5.1)	8 (7.9)
Supraventricular Tachyarrhythmias	20 (6.6)	10 (3.3)	6 (5.8)	0 (0.0)	4 (4.0)
Atrial Fibrillation or Atrial Flutter	15 (5.0)	6 (2.0)	3 (2.9)	0 (0.0)	3 (3.0)
Atrial Tachycardia	3 (1.0)	1 (0.3)	1 (1.0)	0 (0.0)	0 (0.0)
Sinus Tachycardia	1 (0.3)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Supraventricular Tachycardia	2 (0.7)	4 (1.3)	3 (2.9)	0 (0.0)	1 (1.0)
Ventricular Tachyarrhythmias	18 (5.9)	17 (5.6)	8 (7.8)	5 (5.1)	4 (4.0)
Ventricular Arrhythmia	4 (1.3)	0 (0.0)	0 (0.0)	0 (0.0)	0 (0.0)
Ventricular Extrasystoles	1 (0.3)	2 (0.7)	1 (1.0)	1 (1.0)	0 (0.0)
Ventricular Fibrillation	2 (0.7)	1 (0.3)	0 (0.0)	1 (1.0)	0 (0.0)
Ventricular Tachyarrhythmia	0 (0.0)	1 (0.3)	1 (1.0)	0 (0.0)	0 (0.0)
Ventricular Tachycardia	11 (3.6)	13 (4.3)	7 (6.8)	3 (3.0)	3 (3.0)
Extrasystoles	0 (0.0)	1 (0.3)	0 (0.0)	0 (0.0)	1 (1.0)



ATOMIC-AHF: Changes in Heart Rate and SBP

PK Concentration Bin Analysis	Control	OM Conc. Bin 1	OM Conc. Bin 2	OM Conc. Bin 3
OM concentration range (ng/ml)		≥88-200	>200-300	>300-787
Heart Rate (beats/min)				
LS means	-4.3	-4.4	-6.3	-6.5
Difference from control		-0.1	-2.0	-2.3
95% CI		(-1.4, 1.1)	(-3.6, -0.4)	(-3.9, -0.6)
p-value		0.835	0.016	0.008
Linear regression slope	p < 0.0001			
SBP (mmHg)				
LS means	-4.6	-4.4	-4.0	-2.2
Difference from control		0.3	0.6	2.4
95% CI		(-1.2, 1.7)	(-1.2, 2.4)	(0.6, 4.2)
p-value		0.719	0.521	0.009
Linear regression slope	p = 0.0017			

N: number of patients in the bin, n: number of observations in the bin. Heart rate measured by ECG. Control = observations in Placebo + PK below quantification limit. PK bin concentration analysis: repeated measures analysis of covariance. Linear regression slope analysis: repeated measures multiple linear regression.



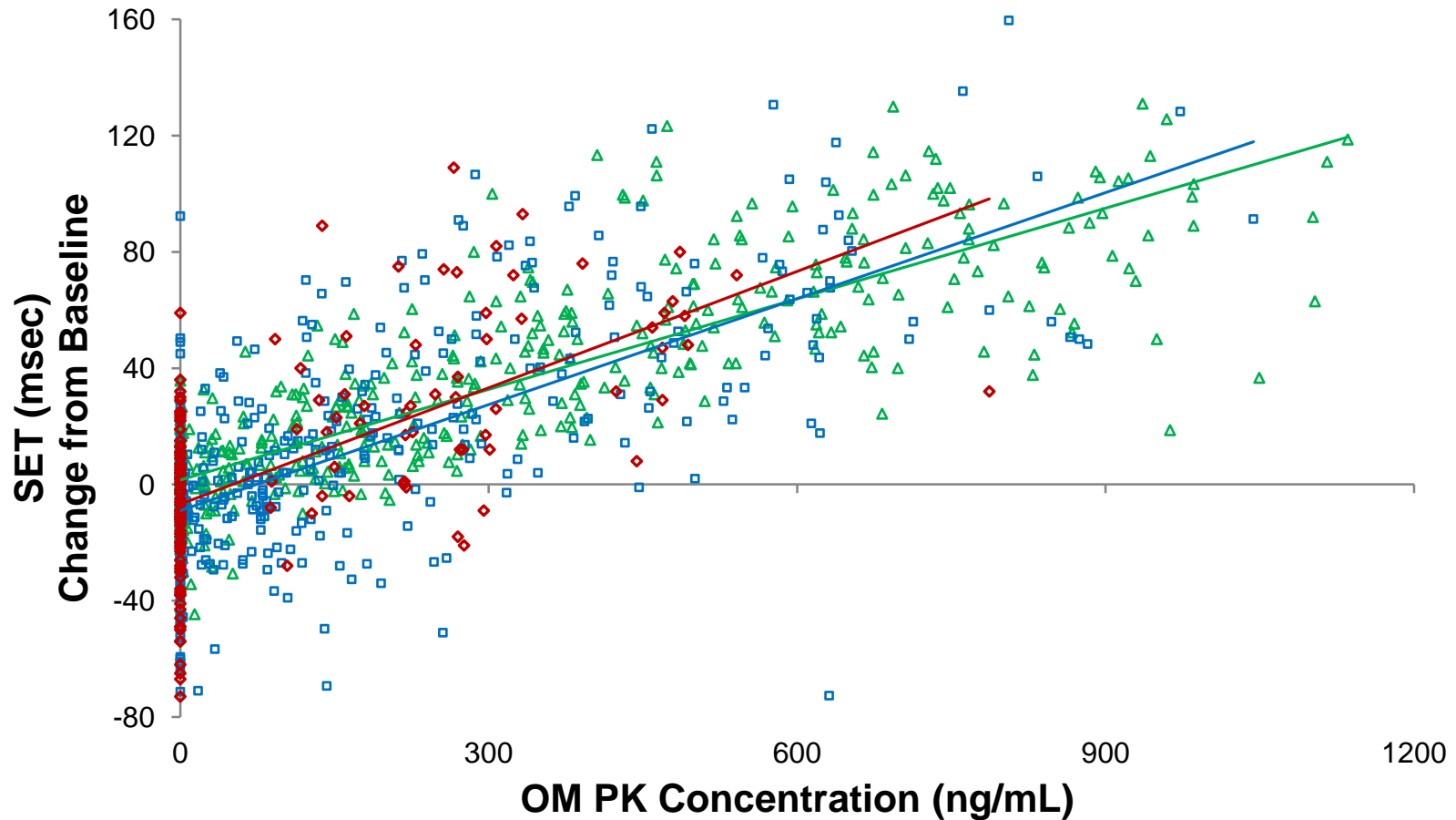
ATOMIC-AHF: Change in Systolic Ejection Time (SET)

PK Concentration Bin Analysis	Control	OM Concentration Bin 1	OM Concentration Bin 2	OM Concentration Bin 3
OM concentration range (ng/ml)		≥88-200	>200-300	>300-787
Change in SET (msec)				
N(n)	45 (88)	10 (18)	15 (23)	12 (19)
LS mean	-6.7	16.6	26.9	46.4
Difference from control		23.4	33.6	53.2
95% CI		(7.4, 39.4)	(19.8, 47.4)	(38.0, 68.3)
p-value		0.005	<0.0001	<0.0001
Linear regression slope	p < 0.0001			

Baseline systolic ejection time for all patients was 258 msec. N: number of patients in the bin, n: number of observations in the bin; Control = observations in Placebo + PK below quantification limit; PK bin concentration analysis: repeated measures analysis of covariance; Linear regression slope analysis: repeated measures multiple linear regression.



Systolic Ejection Time: Volunteers, CHF and AHF Patients



△ Healthy Volunteer

□ Chronic HF

◇ Acute HF

— Linear (Healthy Volunteer)

— Linear (Chronic HF)

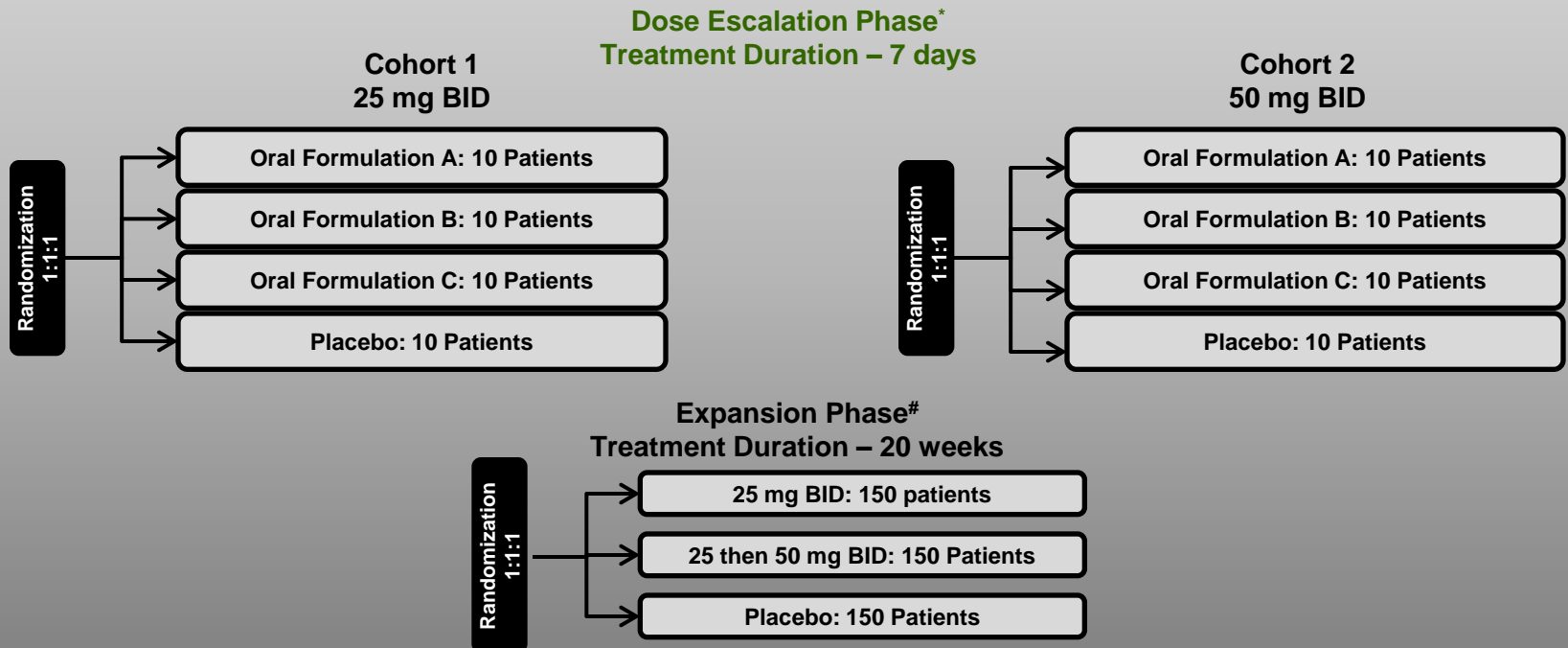
— Linear (Acute HF)



Omecamtiv Mecarbil: COSMIC-HF Phase II Clinical Trial

Chronic Oral Study of Myosin Activation to Increase Contractility in Heart Failure

Multi-center, randomized, double-blind, placebo controlled, dose escalation study to select and evaluate an oral modified release formulation in ~ 530 Chronic Patients with Heart Failure and Left Ventricular Systolic Dysfunction



#Expansion phase follows completion of escalation phase



CORPORATE



Skeletal Muscle Program: Astellas Collaboration

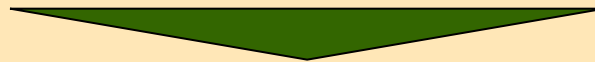
Cytokinetics eligible to receive over \$40 mm in upfront payments and reimbursement of sponsored research and development activities



- **Astellas responsible for activities and costs associated with development products**
- **Cytokinetics retains an option to conduct early-stage development for certain indications at its expense, subject to reimbursement if development continues**



Cytokinetics eligible to receive over \$450 mm in pre-commercialization and commercialization milestones plus royalties



- **Astellas will have exclusive right to commercialize collaboration products worldwide subject to Cytokinetics' option to co-promote collaboration products in the US and Canada**
- **Astellas will reimburse Cytokinetics for certain expenses associated with its co-promotion activities**



Omecamtiv Mecarbil: Amgen Collaboration

Amgen paid \$75 mm
for option exercisable on Phase IIa clinical trials program
(December 2006)

Amgen paid \$50 mm to exercise option
for exclusive rights (ex-Japan) (May 2009)

Amgen paid \$25 mm to expand license
to include Japan and purchase equity
(June 2013)

- Amgen responsible for development and commercialization subject to Cytokinetics' participation rights
- Cytokinetics can earn up to \$650 mm in milestone payments

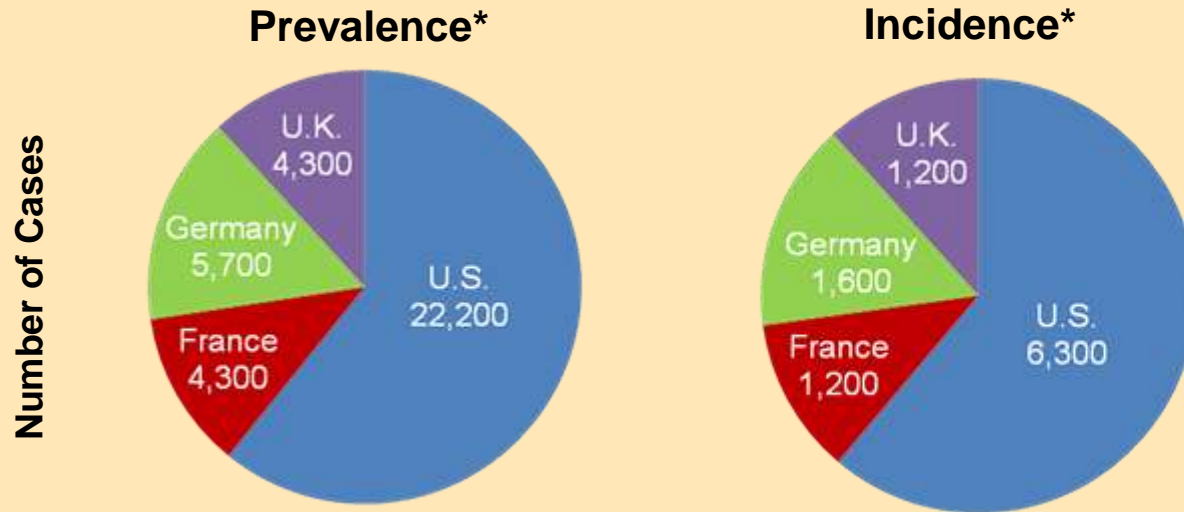
Commercialization

- Cytokinetics to receive escalating double-digit royalties
- Increased royalties through co-funding Phase III trials
- Option to co-promote co-funded products in NA
- Cytokinetics reimbursed for certain sales force costs



Tirasemtiv: High Unmet Need in ALS

Incidence and Prevalence of ALS



- No medicine available to address functional impairment
- Most common motor neuron disease in adults
- Despite the significant incidence of the disease, prevalence remains relatively low due to the rapid progression to death

An Orphan Population with Urgent Unmet Medical Needs



ALS Centers of Excellence: Concentrated Market



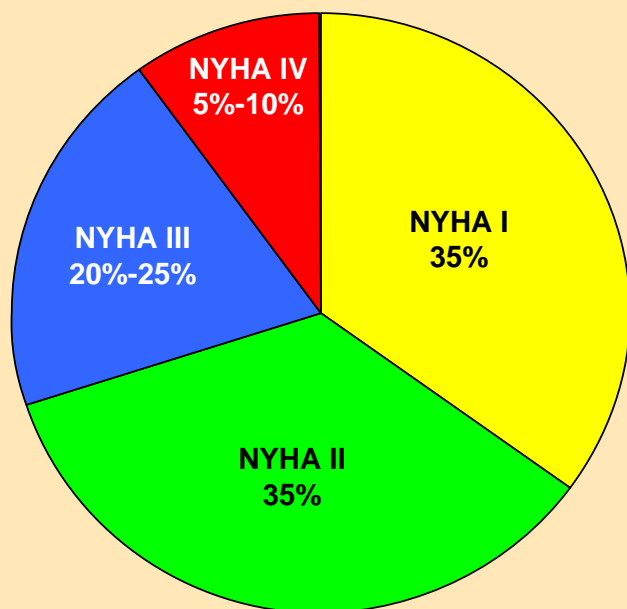
- **Multidisciplinary:** Comprehensive care from experienced ALS team
- **Concentrated:** Majority of ALS patients are treated at a Center of Excellence
- **Regular Visits:** Patients are typically seen by team every 3-6 months

Concentrated Target Audience for Novel Therapies



Omecamtiv Mecarbil: High Unmet Need in Heart Failure

US Population by NYHA Class Total = ~5 million

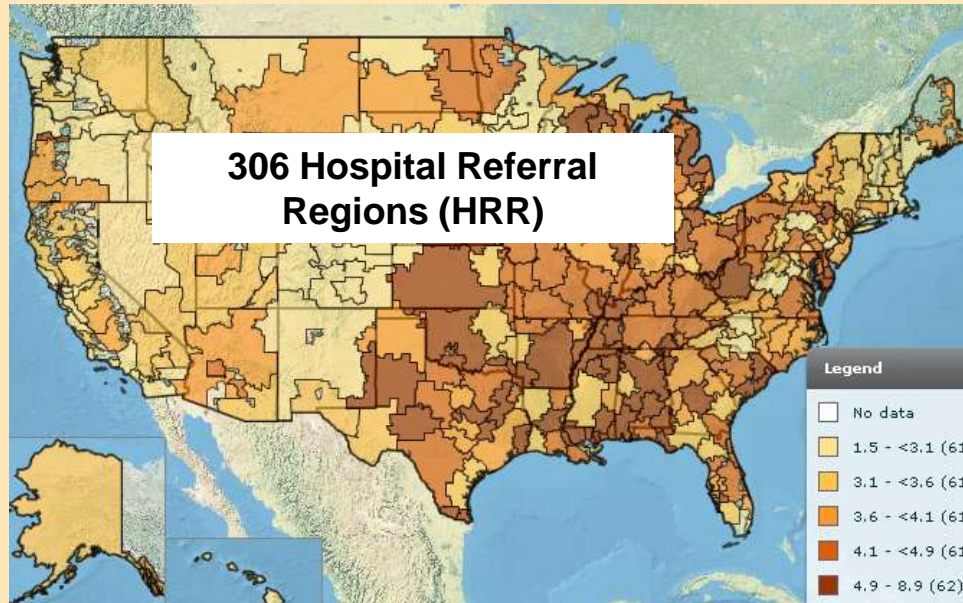


High Unmet Need

Acute	Chronic
Opportunity for novel mechanism, evidence-based therapies to reduce mortality	
Lack of treatment and follow-up in patients to reduce the rate of hospital readmission	
Absence of treatments to increase cardiac performance without accompanying increases in heart rate and arrhythmias	



Medicare Hospitals for Heart Failure: Concentrated Market



2010 Medicare Population

- ~4.5 million Medicare beneficiaries with heart failure
- ~50% of Medicare beneficiaries with heart failure concentrated in 59 HRRs (20% of HRRs)
- ~75% of Medicare beneficiaries with heart failure concentrated in 131 HRRs (43%) of all HRRs

Concentrated Target Audience for Novel Therapies



Q1 Condensed Balance Sheet Data

	<u>3/31/2014</u> (in millions)
Cash, cash equivalents and investments*	\$101.9
Other assets	5.4
Total assets	107.3
Deferred revenue, current & long term	11.2
Other liabilities	9.9
Total liabilities	21.1
Working capital	66.3
Accumulated deficit	(491.3)
Total stockholders' equity	86.2
Total shares outstanding	36.1

* Includes \$37.4M from February 20, 2014 Public Offering



Current 2014 Financial Guidance*

	(in millions)
Cash Revenue	\$19- \$21
Cash R&D Expenses	\$50 - \$53
Cash G&A Expenses	\$15 - \$17

*This guidance is on a cash basis and does not include approximately \$10 million in revenue deferred from 2014 under GAAP nor an estimated \$3 million in non-cash related operating expenses primarily related to stock compensation expense.

In addition, this guidance does not reflect potential revenues from milestone payments that may be achieved in partnered programs.



Capitalization Table

Basic Shares Outstanding @ 3/31/14	36,090,071
Warrants 2011 @ \$9.90	1,114,168
Warrants 2012 @ \$5.28	6,576,928
Employee Stock Plans	
Issued & outstanding	3,269,661
Available for grant	1,361,973
Stock Purchase Plan Reserve	189,896
Fully Diluted Outstanding	48,602,697



Progress in 2013 Reinforces Outlook for 2014

- **Advancing multiple muscle biology directed programs**
- **Achieved clinically relevant effects in Phase IIb trials**
- **Transacted corporate development deals to diversify risk**
- **Positioned well for continued progress in late stage development**
- **Strong balance sheet and diversified funding sources**
- **Continued commitment to patients with severe diseases**





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