



Baird Global Healthcare Conference Presentation

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Lead program

BLU-5937

for chronic cough

Large population with high
unmet need

Clinically validated target

Clear and efficient
development path

Phase 1 on-going with data
in Q4 2018

Listed on the Toronto Stock Exchange

TSX - BLU

Experienced

management with track record of execution

2+

years runway through multiple clinical
milestones

Generating value by advancing drug candidates through clinical studies

Management



Roberto Bellini
President &
Chief Executive Officer



Dr. Denis Garceau
Senior Vice President,
Drug Development



François Desjardins
Vice President,
Finance



Tony Matzouranis
Vice President,
Business Development

Board of Directors



Dr. Francesco Bellini
(Chair)



Franklin Berger



Pierre Larochelle



Dr. Youssef Bennani



Joseph Rus



Dr. Clarissa Desjardins



Roberto Bellini

Management with a track record of execution

Problem: Refractory Chronic Cough

Cough lasting
≥8 weeks,
0 therapies that are
safe **and** effective

Major
impact on quality
of life

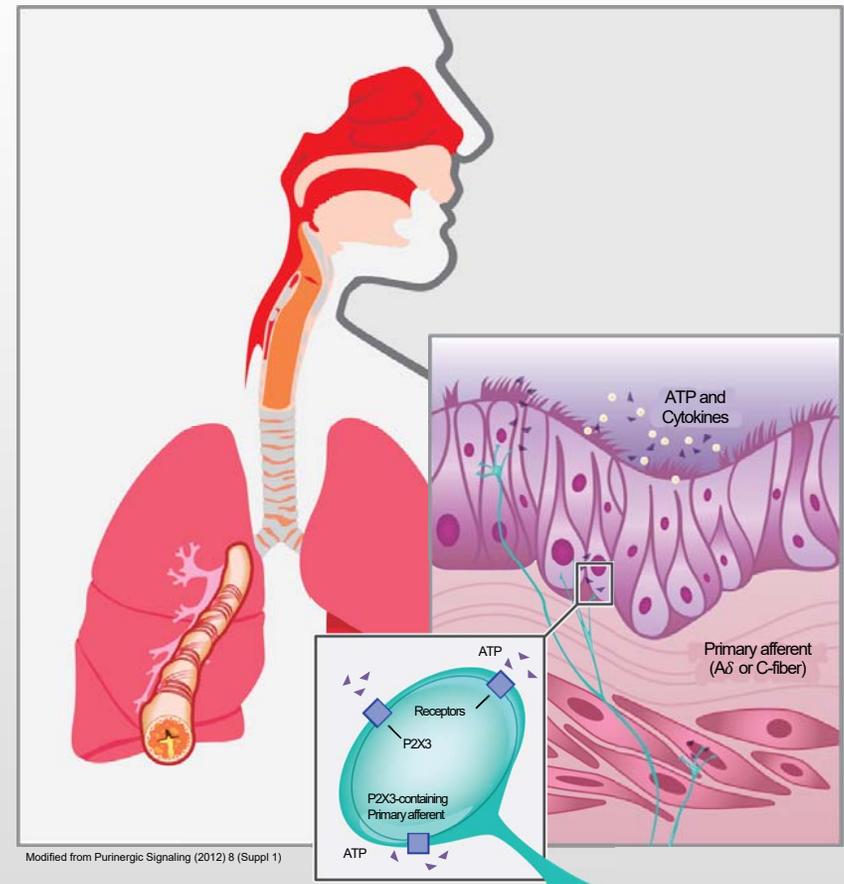
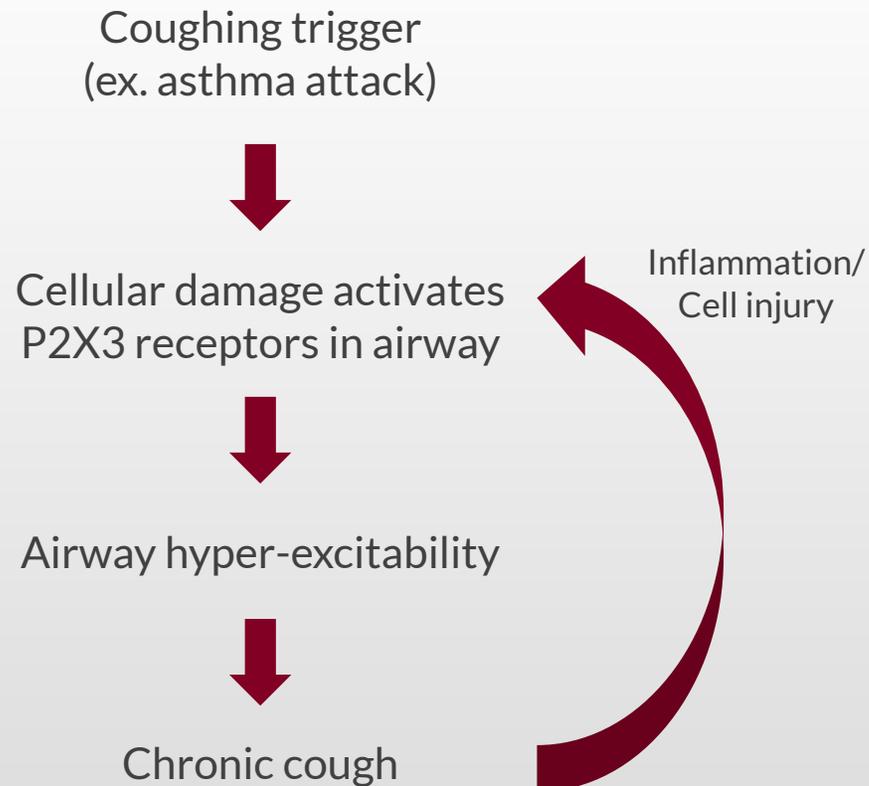
“I see patients that have been coughing 2 months to 30 years. Within that group, there is a good portion where I am the 8th or 10th doctor.”

– Chronic Cough KOL

2.6M
patients in U.S. with longstanding
refractory chronic cough

Multi \$B
market potential

Cause: Hypersensitive Cough Reflex



P2X3 is a sensory receptor found in peripheral nervous system with central role in triggering cough reflex

Treatment in Development is Suboptimal



Effective

Reduces awake cough frequency by

86%



Mechanism:
P2X3
antagonist

Major Side Effect

80%

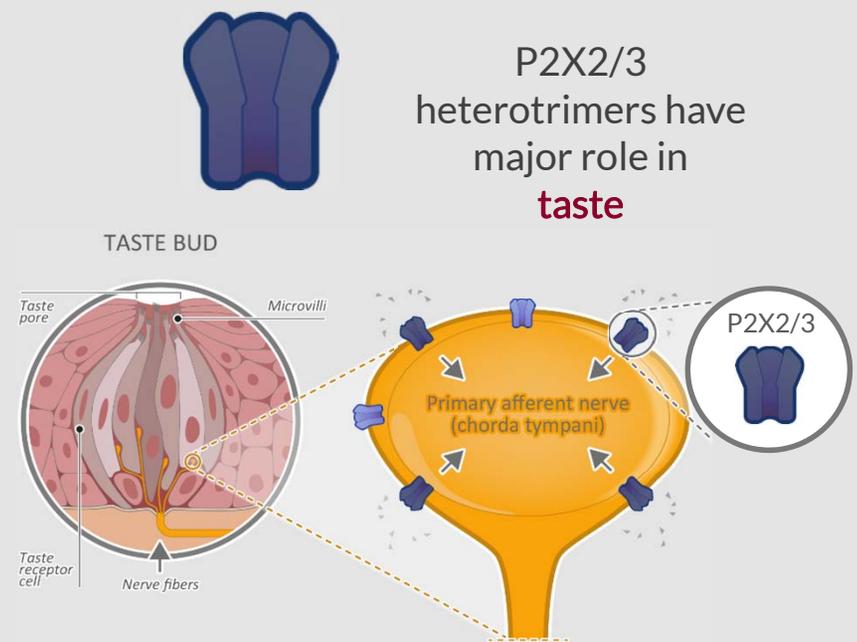
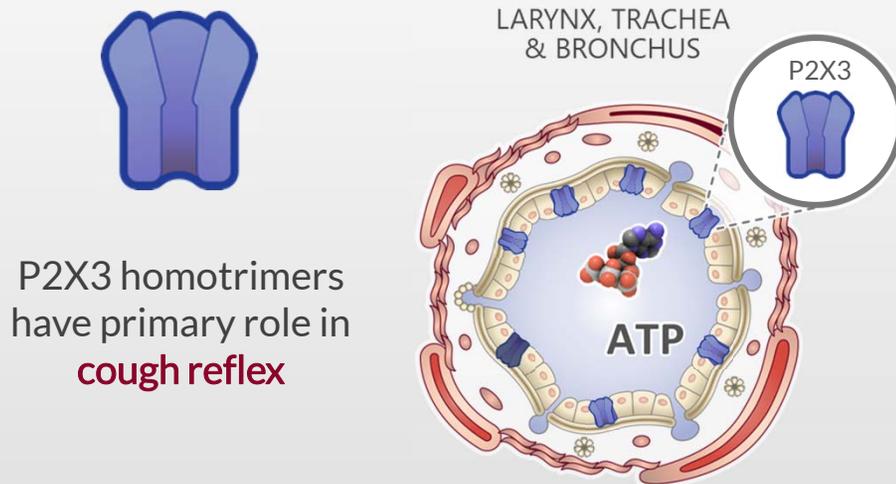
of patients have taste alteration or taste loss

Merck & Co., Inc. (2017). *Merck Announces Presentation of Phase 2 Results for MK-7264, an Investigational, P2X3 Receptor Antagonist, Being Evaluated for the Treatment of Chronic Cough.* [Press Release]. Retrieved from <http://www.mrknewsroom.com/news-release/research-and-development-news/merck-announces-presentation-phase-2-results-mk-7264-inve>

Acquired in 2016 for \$1.25B (\$500M upfront) based on phase 2 data

MK-7264 Effect on Taste Likely Caused by Inhibition of P2X3 and P2X2/3

P2X3 and P2X2/3 are ATP-gated ion channels that transmit sensory signals:



Project hypothesis: Opportunity for highly selective P2X3 antagonist to reduce cough, maintain taste (no P2X2/3 inhibition)

Equivalent
reduction in
cough frequency



Highly selective
P2X3
antagonist

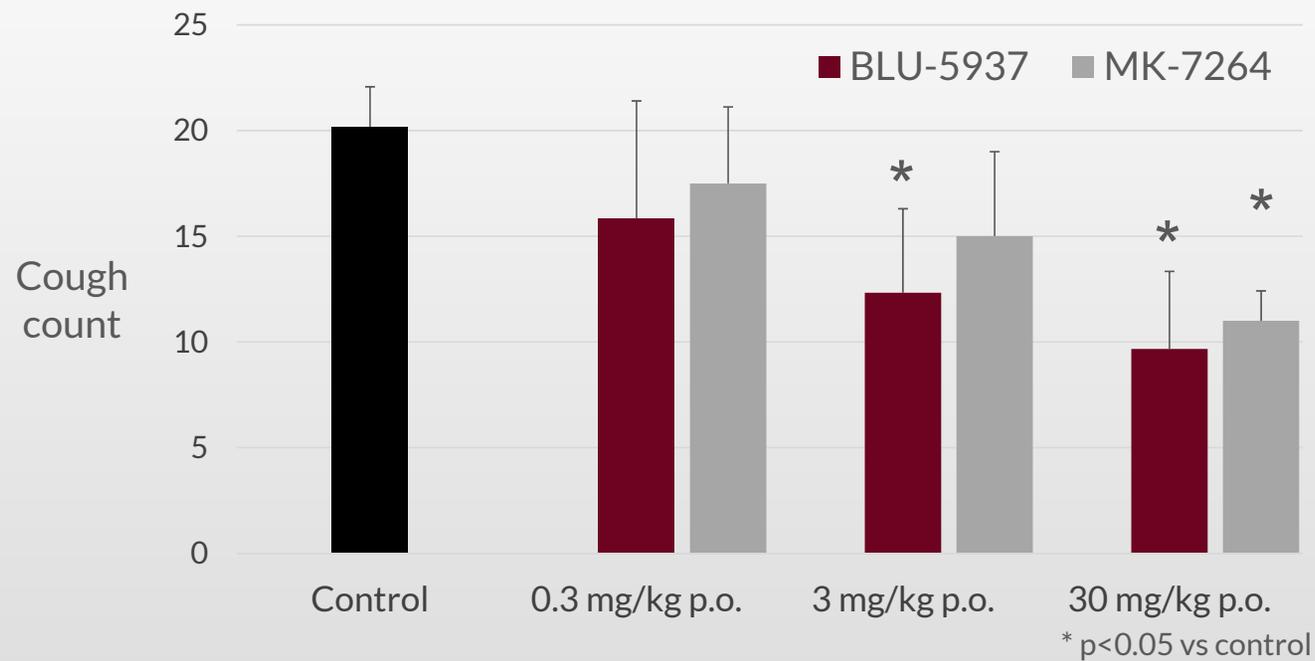
No
impact on taste

vs. MK-7264 in animal studies

Project hypothesis validated in animal studies

Cough Inhibition in Guinea Pig Model

Cough Response Study

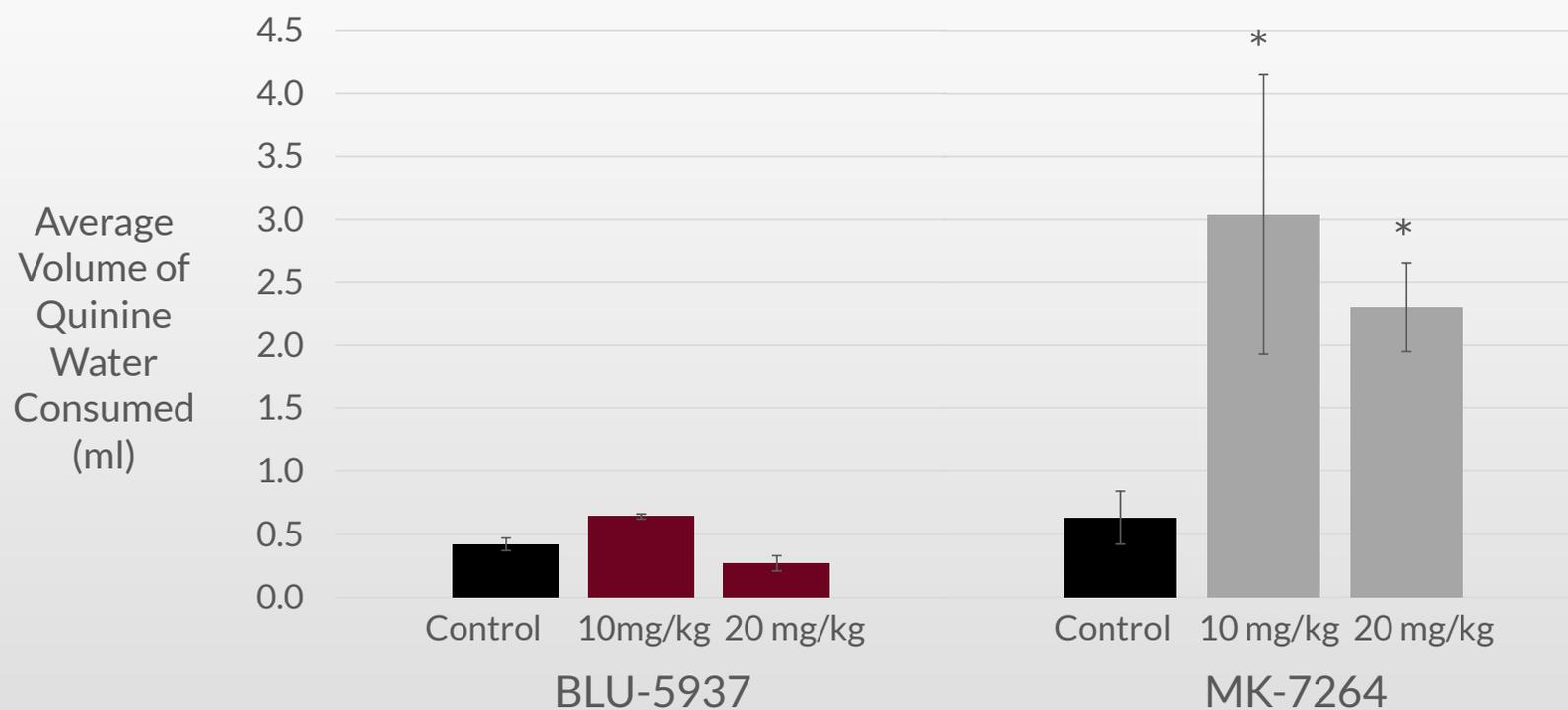


Treatments (control, BLU-5937, MK-7264) were administered orally (p.o.) 2 hours prior to tussive agent exposure: citric acid (0.1 M, aerosol) and histamine (0.6 mM, aerosol); n=6 animals per group

BLU-5937 inhibits cough dose dependently and comparably to MK-7264

Taste Effect in Rat Taste Model

Two Bottle Rat Taste Study



Treatments (control, BLU-5937, MK-7264) were administered ip; animals were water-fasted overnight and presented with one bottle of water and one bottle of water plus quinine (0.3mM) at T_{max} ; volume of liquid consumed measured for 15 minutes; n=10 animals per group; * p < 0.05 vs control

MK-7264 changes taste perception; BLU-5937 does not

BLU-5937: Potential Best-in-Class Profile



Twice Daily
Oral Dosing
Expected

High
Selectivity and
Potency for P2X3

No
safety findings
of concern

Broad and
comprehensive IP to
2034

Targeting
~2.6M
US Patients

Strong drug candidate profile with potential to be best in P2X3 class

P2X3 Antagonist Competitive Landscape



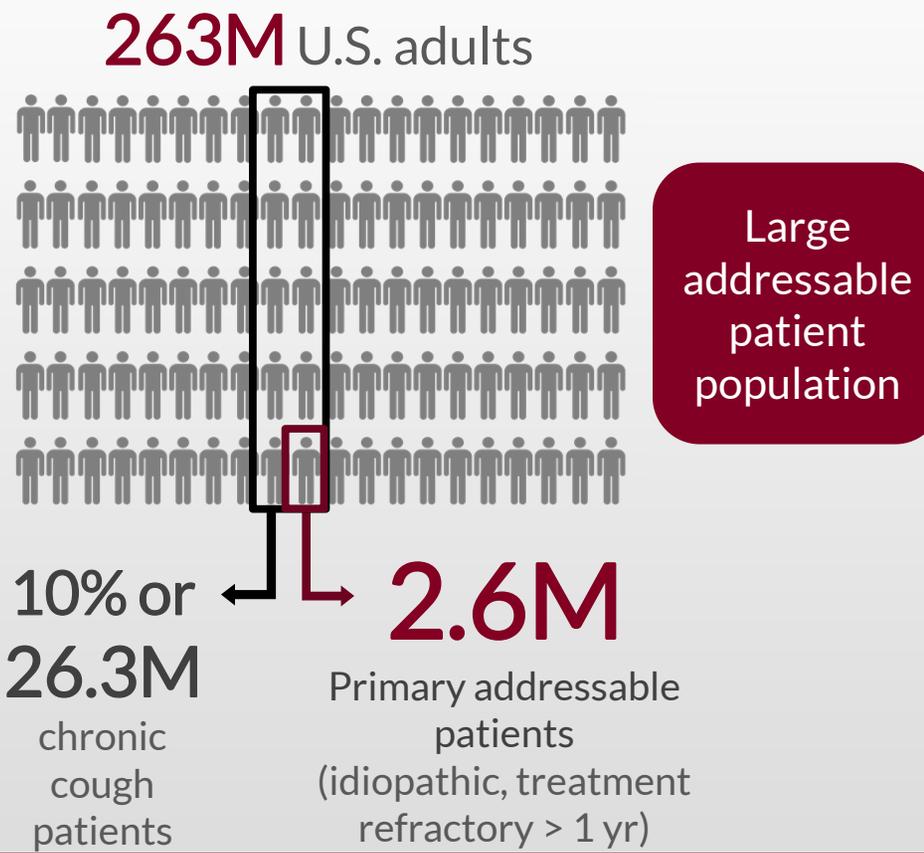
	Merck MK-7264	Merck MK-7264	Bayer BAY 1817080	BELLUS BLU-5937
Phase	3	3	2	1
Dosing	15 mg B.I.D.	45 mg B.I.D.	B.I.D.	B.I.D.
Selectivity for P2X3 (vs. P2X2/3)	2 - 7 x	2 - 7 x	25 - 125 x	> 2000 x
Anti-tussive effect ¹	Low	High	High	High
Effect on taste ¹	Low	High	Moderate/Low	Low/None

Best in class selectivity for P2X3 supports potential best in class profile ←

¹Effect on taste and anti tussive effect are company estimates based on animal data, clinical data, dose, human P2X3 potency and human P2X3 vs P2X2/3 selectivity

BLU-5937 is potential best-in-class profile in large, big pharma validated, cough market

Market



Comparable products



Payer discussions and comparable product analysis support \$300-600 per month pricing

BLU-5937: addressing potential multi billion dollar refractory chronic cough market₁₄

Key Development Milestones



File clinical trial application

Start Phase 1

Phase 1 data

Start Phase 2

Q2 2018

Q3 2018

Q4 2018

2019

Phase 1 enabling studies
Complete

Phase 1 design
Study Initiated

Effect on taste
Safety/tolerability
Dose selection for Phase 2

Effect on cough and taste
Dose selection for Phase 3

Efficient development plan with short term value inflection points

Phase 1 Study Design

Key Objectives

Assess Safety

Assess Tolerability
including taste
effect

Measure Drug
Plasma Levels
for Phase 2
dosing

Single
Ascending
Dose

N ≈ 60 healthy adult subjects
~6 cohorts of 10 subjects
(8 active: 2 placebo)
administered single dose

N ≈ 30 healthy adult subjects
~3 cohorts of 10 subjects (8 active: 2
placebo) administered multiple dose
for 7 days

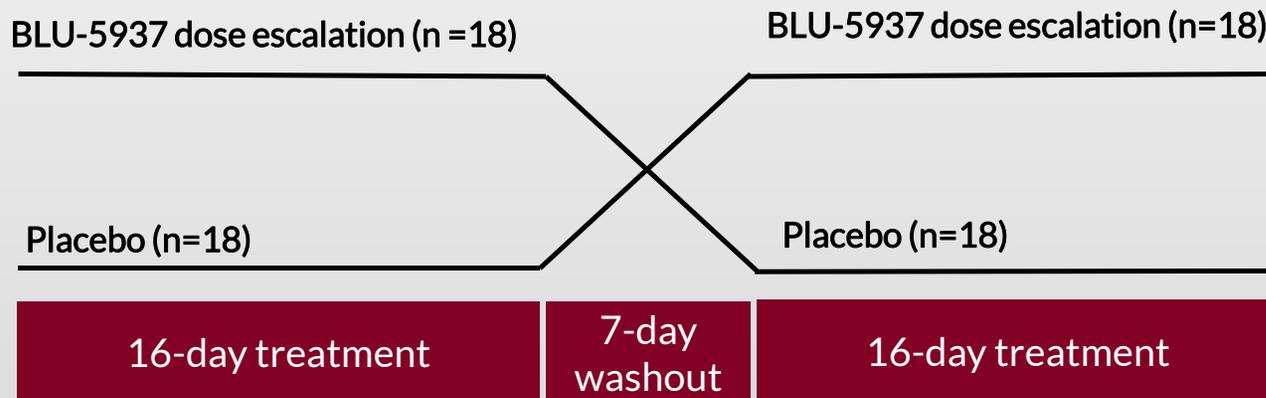
Dose regimen selected based on
single dose portion of study

Multiple
Ascending
Dose

Phase 1 data expected in Q4 2018

Potential Phase 2 Proof of Concept Study Design

- N≈36 unexplained/refractory chronic cough patients; >1 year coughing
- Sites in UK and US
- 4 dose levels escalated at 4-day intervals
- Primary endpoint: Reduction in awake cough frequency
- Safety and tolerability assessment, including taste effect



Design to be further refined based on Phase 1 data and input from clinical advisory board. Expected start in 2019.

Additional Partnered Programs in Pipeline



Program	Partner	Indication	Stage	Next Steps
KIACTA™	AUVEN THERAPEUTICS	Sarcoidosis	Phase 2 ready	Auven evaluating partnering options to finance/conduct Phase 2 study
AMO-01	AMO PHARMA	Phelan McDermid Syndrome	Phase 2	Phase 2 initiated in Q2 2018
ALZ-801	ALZHEON preserving future memories	Alzheimer's disease	Phase 3 ready	Alzheon preparing for Phase 3 in APOe4 homozygous patients

Three mid-stage partnered projects with revenue share and/or royalty potential

Stock and Financial Information



Diversified shareholder base

with significant healthcare-
focused institutional
ownership

~35%	~30%
institutional ownership	family offices

Insider reporting, NOBO list and company estimates

Clean capital structure

119.5M basic shares
132.7M fully diluted shares
~C\$115M market capitalization /
US\$80M market capitalization

C\$20.2M / US\$15.5M

cash position¹ provides

2+

years of runway through Phase 1 and Phase 2

¹as at June 30, 2018

Current cash runway provides 2+ years of capital through multiple milestones



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