



Corporate Presentation

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President and Chief Executive Officer
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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

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



PICCHIO
INTERNATIONAL

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

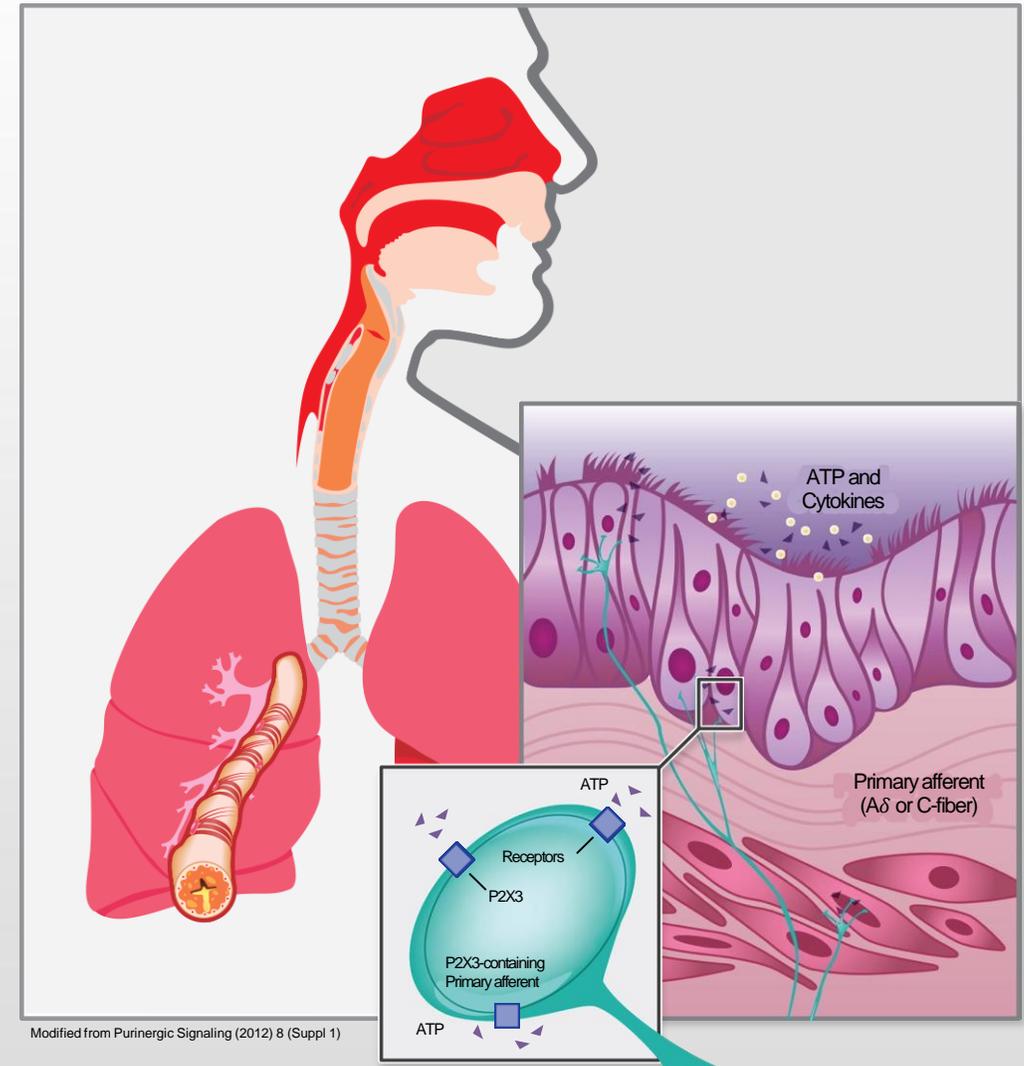
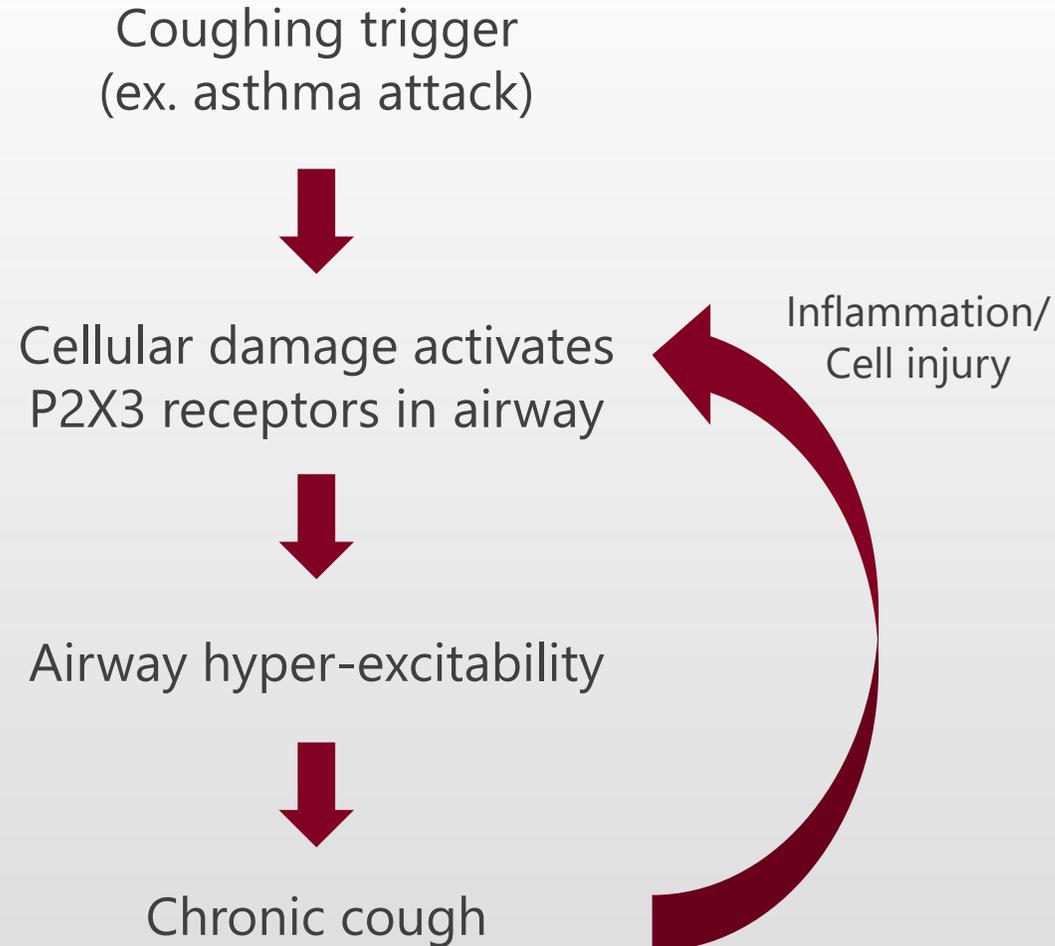
"I see patients that have been coughing 2 months to 30 years. Within that group, there is a good portion where I am the 8th or 10th doctor."

– 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>

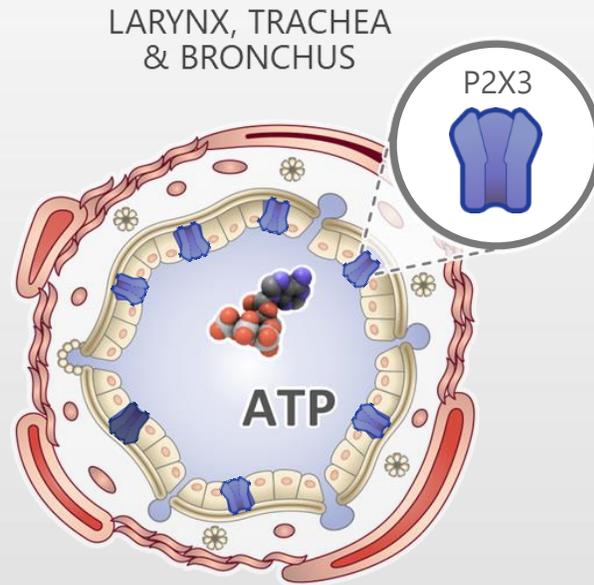
Acquired in 2016 for \$1.25B (\$500M upfront) based on phase 2 data

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

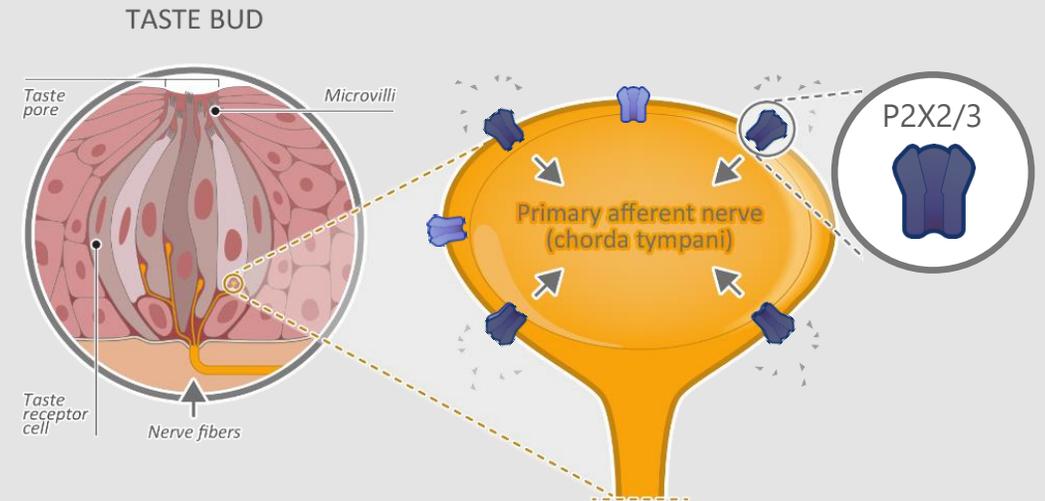
P2X3 and P2X2/3 are ATP-gated ion channels that transmit sensory signals:



P2X3 homotrimers have primary role in **cough reflex**



P2X2/3 heterotrimers have major role in **taste**



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

Equivalent
reduction in
cough frequency



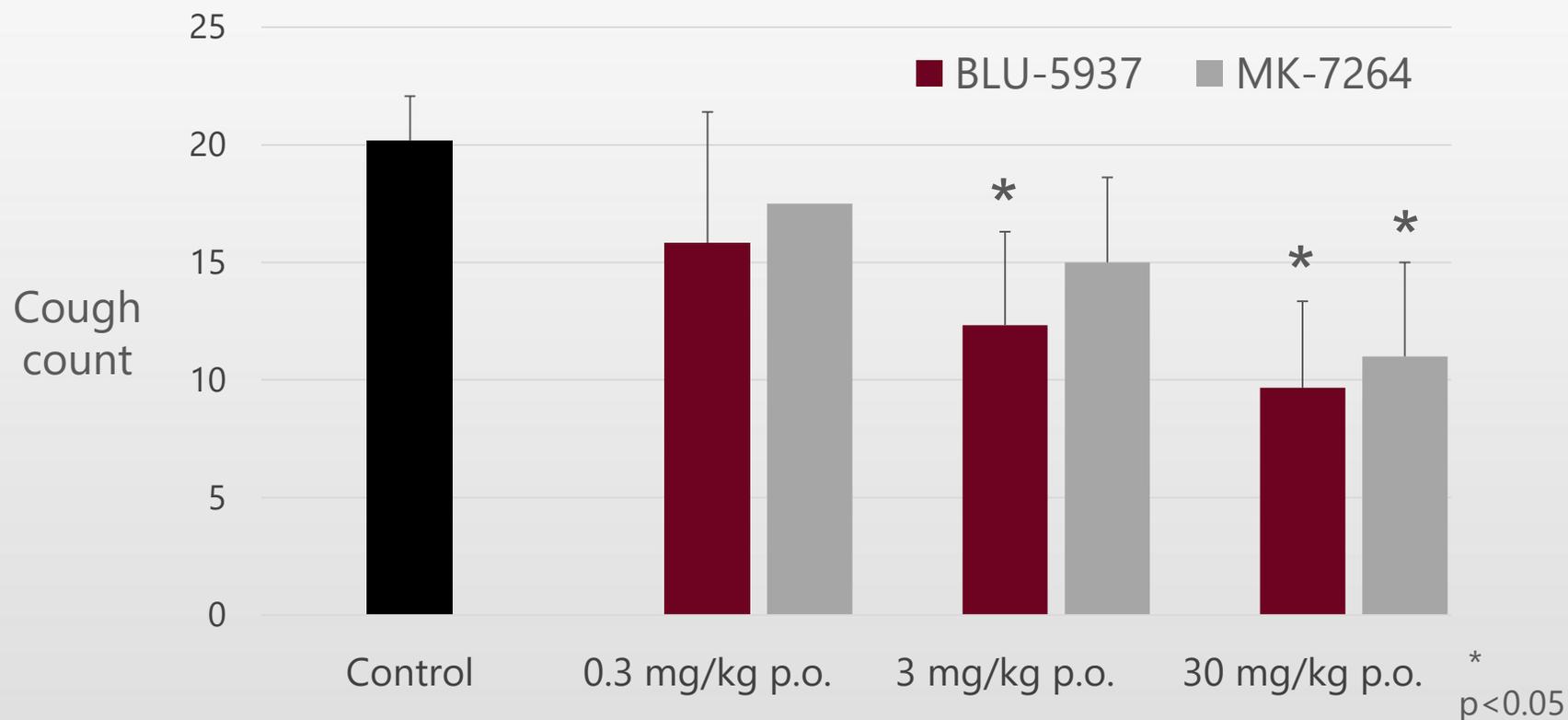
Highly selective
P2X3
antagonist

No
impact on taste

vs. MK-7264 in animal studies

Cough Inhibition in Guinea Pig Model

Cough Response Study

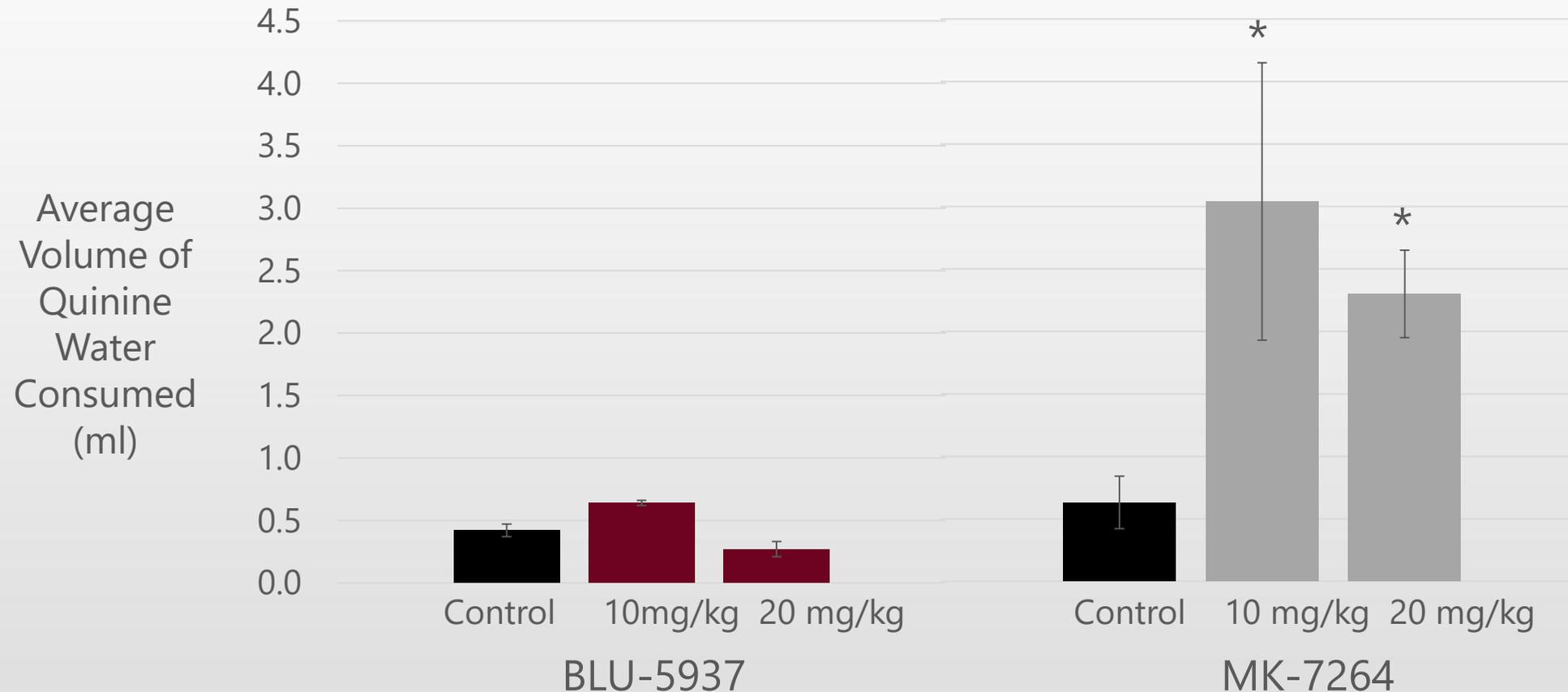


Treatments (control, BLU-5937, MK-7264) were administered orally (p.o.) 2 hours prior to tussive agent exposure: citric acid (0.1 M, aerosol) and histamine (0.6 mM, aerosol); n=6 animals per group

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

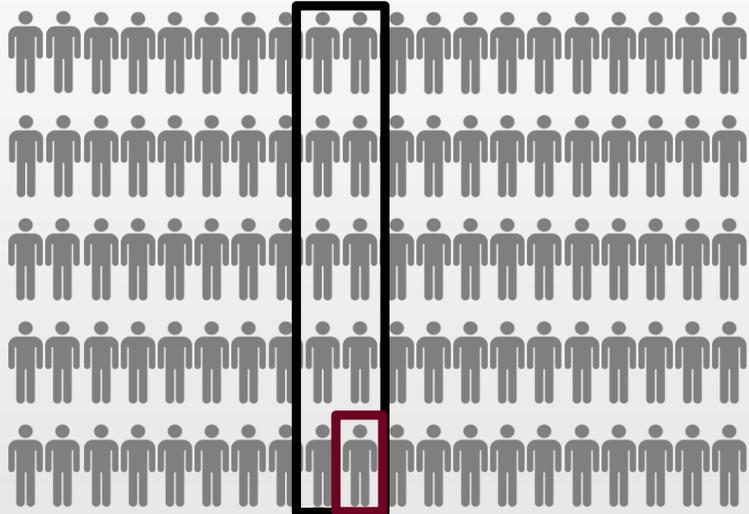
No
safety findings
of concern

Broad and
comprehensive IP to
2034

Targeting
~2.6M
US Patients

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

263M U.S. adults



Large addressable patient population

10% or
26.3M

chronic cough patients

2.6M

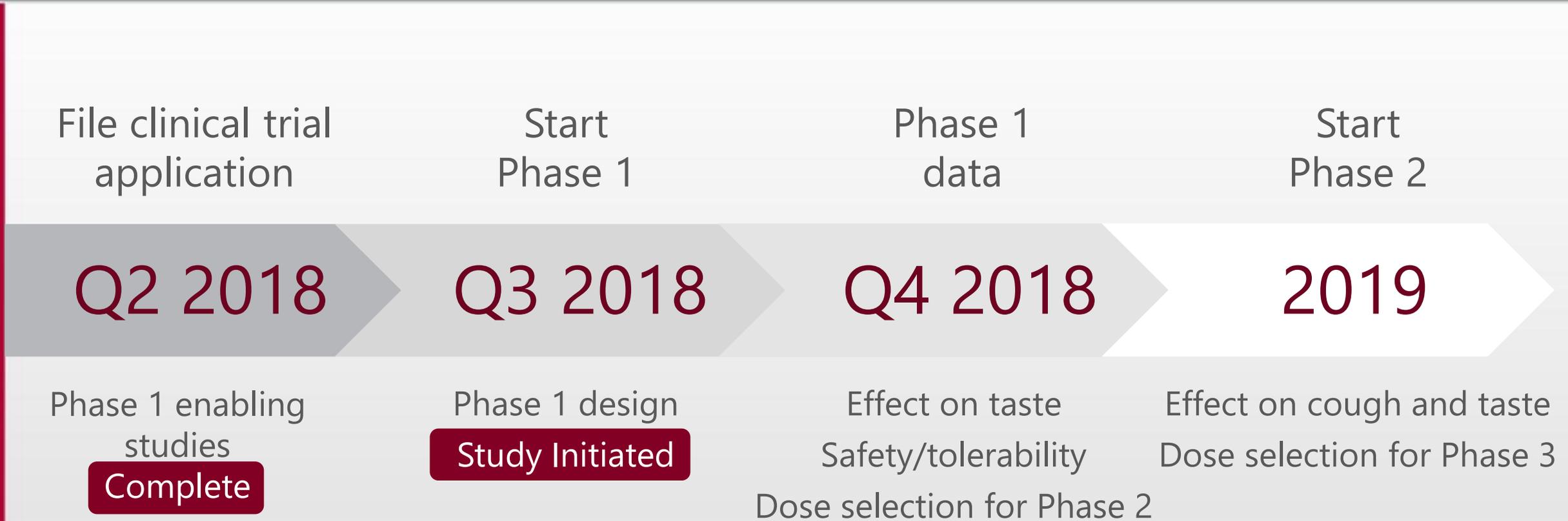
Primary addressable patients (idiopathic, treatment refractory > 1 yr)

Comparable products



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

Key Development Milestones



Efficient development plan with short term value inflection points

Key Objectives

Assess Safety

Assess Tolerability
including taste
effect

Measure Drug
Plasma Levels
for Phase 2
dosing

Single
Ascending
Dose

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

N \approx 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 designed to assess safety, tolerability (including taste effect) and drug levels

Additional Partnered Programs in Pipeline

Program	Partner	Indication	Stage	Next Steps
KIACTA™	 <p>AUVEN THERAPEUTICS</p>	Sarcoidosis	Phase 2 ready	Auven evaluating partnering options to finance/conduct Phase 2 study
AMO-01	 <p>AMO PHARMA</p>	Phelan McDermid Syndrome	Phase 2	Phase 2 initiated in Q2 2018
ALZ-801	 <p>ALZHEON preserving future memories</p>	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

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
~\$65M market capitalization

\$21.7M

cash position¹ provides

2+

years of runway through Phase 1 and Phase 2

¹as at March 31, 2018

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



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