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Meaningfully Improving the Lives of Patients with Rare Cardiopulmonary Disease

Targeting the Hyperproliferative Cause of Pulmonary Arterial Hypertension

October 2021

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Experienced Management Team

Timothy Noyes

Chief Executive Officer

- 30 years' industry experience in pharma and biotech, with both public and private companies, including Merck, Genzyme, Proteon
- Extensive launch planning and commercial launch experience

George Eldridge

Chief Financial Officer

- 25 years' experience in biotech, with both public and private companies, including Curis, Targanta, Proteon
- Extensive background raising capital in private, IPO and follow-on settings, investment banking and M&A

Ben Dake, PhD

President & Founder

- Entrepreneur, Cancer Biologist and Investor
- Conceptualized AV-101 and secured up to $79M in financing for Aerovate

Ralph Niven, PhD

Chief Development Officer

- 25 years' broad expertise in translational medicine and inhalation dosage forms
- Managed experimental and clinical development at public and private companies, including Amgen, AIR and Novartis

Hunter Gillies, MB ChB

Chief Medical Officer

- Led AMBITION trial for Gilead that established current first-line PAH combination therapy
- Led successful Phase 2 and 3 trials for PAH product candidates at Pfizer and Gilead
- Designed and executed several POC PAH patient trials in biotech

Timothy Pigot

Senior Vice President, Commercial

- 25 years' experience in biotech and pharma working to launch and commercialize a range of products
- 10 years' experience in PAH overseeing the US launches of Revatio and Leairis while at Pfizer and Gilead Sciences, respectively

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Aerovate: An Inventive Way Forward in PAH

Demonstrated Clinical Benefit

The molecule in AV-101, imatinib, already has shown clinical benefit in a Phase 3 clinical trial conducted by Novartis of oral imatinib mesylate in PAH patients on top of two or more standard of care therapies.

Unfortunately, AEs with oral imatinib were common and development was discontinued

Inventive Solution

Anti-proliferative targeted inhaled dry powder PAH product candidate AV-101 designed to provide robust clinical benefit of imatinib without systemic AEs observed with oral imatinib

Significant Unmet Need

Despite three drug classes approved, 5-year survival for newly diagnosed PAH patients is 61%

Efficient Execution

FDA and EMA orphan designation for AV-101 for the treatment of PAH

Phase 1 SAD/MAD in healthy volunteers complete

Phase 2b dose-ranging portion of a Phase 2b/3 trial in PAH patients anticipated to start in H2 2021

Established Market

$5B+ market, yet outcomes are poor

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Relentless Disease Progression Impairs Daily Life

No limitation

Activity limited

Comfortable at rest, but ordinary physical activity causes shortness of breath, fatigue, chest pain, fainting

Marked limitation

Comfortable at rest, but less than ordinary physical activity causes shortness of breath, fatigue, chest pain, fainting

Severe limitation

Symptoms at rest. Overt heart failure

Despite standard of care (SOC) most patients progress to overt heart failure

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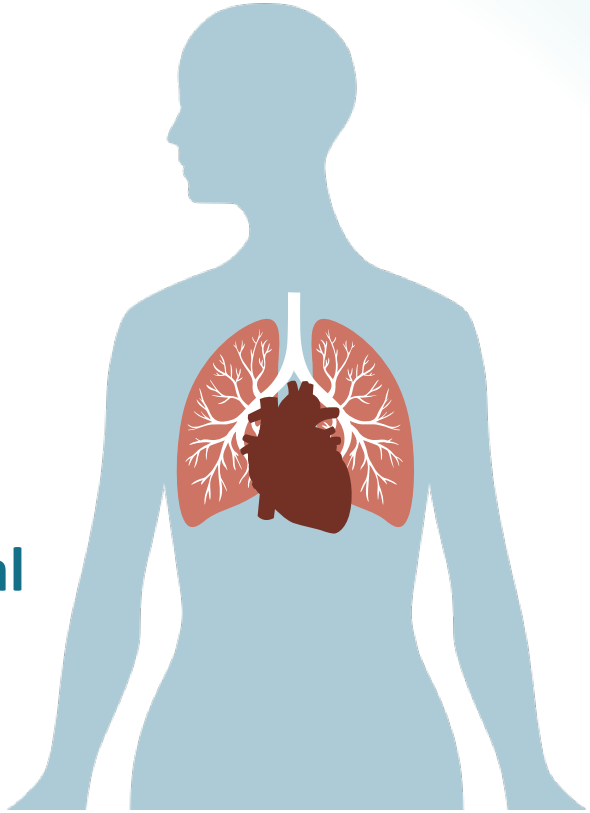
Pulmonary Arterial Hypertension

~ 70,000 People with PAH in the US/EU	**~ 35,000** People with PAH in the US	**$5B+** Global annual drug spending
65-80% Female	**53 years** Average age at diagnosis	**61-65%** **5-year survival** Newly diagnosed and prevalent patients



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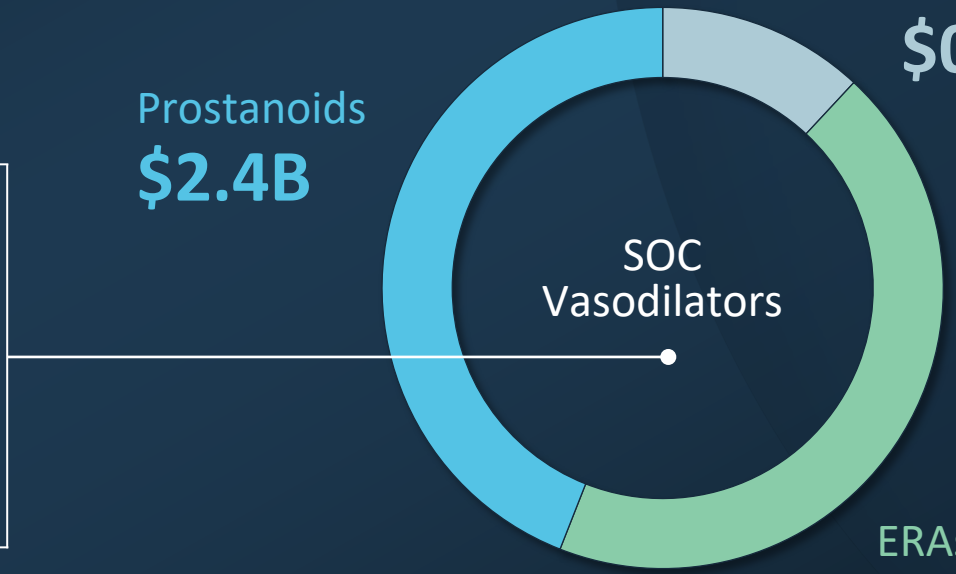
Patient Outcomes Are Poor Despite $5B+ PAH Vasodilator Market

~50% patients take
2-3 drugs

5-year survival
61-65%
for newly diagnosed and prevalent patients



No approved PAH therapy primarily addresses abnormal cellular proliferation

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Reinventing Imatinib From a Cancer Drug to a Potential PAH Therapy

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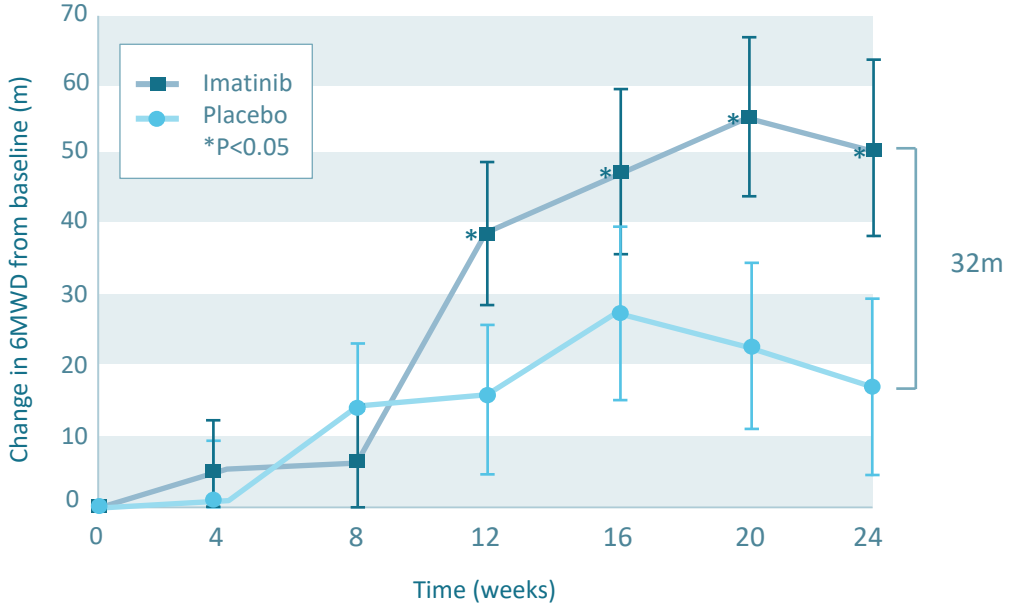
Phase 3 IMPRES Trial: Oral Imatinib Demonstrated Improvement on Top of Maximal Background

- Novartis global Phase 3 trial (n=202) of oral imatinib
- Required to be on at least 2 SOC PAH drugs
- WHO functional class II- IV
- Statistically significant and clinically meaningful benefit on primary endpoint 6MWD after 24 weeks
- 6MWD is an accepted endpoint for approval in PAH



(Adapted from Circulation. 2013;127:1128-1138.)

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Phase 3 IMPRES Trial: Benefit Consistent Across Secondary Endpoints

Secondary Endpoints (24wk)

Demonstrated robust hemodynamic effect

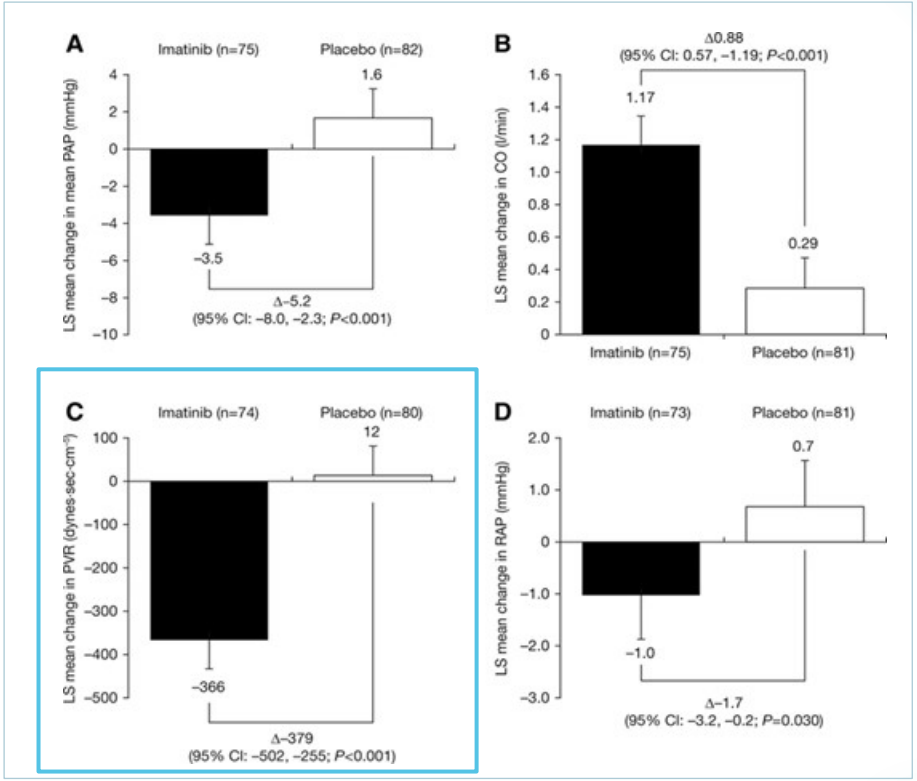
(A) Decreased mean pulmonary artery pressure

(B) Increased cardiac output

(C) Pulmonary vascular resistance (PVR) dropped 32% (P < 0.001), a validated efficacy endpoint typically used for dose-finding

(D) Right atrial pressure lowered

Aerovate's Phase 2b trial will use PVR as the primary endpoint



(Circulation. 2013;127:1128-1138.)

Oral Imatinib Caused Systemic Adverse Events

	Imatinib n=103 (%)	Placebo n=98 (%)
Adverse Event +	100 (97)	94 (96)
Nausea	57 (55)	23 (24)
Peripheral edema	45 (44)	20 (20)
Diarrhea	36 (35)	19 (19)
Vomiting	31 (30)	10 (10)
Periorbital edema	30 (29)	7 (7)
Headache	25 (24)	22 (22)
Dyspnea	19 (18)	13 (13)
Nasopharyngitis	18 (18)	19 (19)
Hypokalemia	16 (16)	3 (3)
Anemia	14 (14)	3 (3)
Cough	11 (11)	15 (15)
Fatigue	11 (11)	7 (7)
Face edema	10 (10)	1 (1)
Muscle spasms	10 (10)	2 (2)

	Imatinib n=103 (%)	Placebo n=98 (%)
Serious Adverse Event +	45 (44)	29 (30)
Worsening of pulmonary hypertension	6 (6)	8 (8)
Anemia	7 (7)	1 (1)
Dyspnea	6 (6)	2 (2)
Peripheral edema	6 (6)	0
Presyncope	5 (5)	0
Diarrhea	3 (3)	2 (2)
Device-related infection	3 (3)	0
Syncope	1 (1)	5 (5)
Subdural hematoma *	2 (2)	0

* In long-term extension portion of the trial, 6 additional patients experienced a subdural hematoma.

+ Individual adverse events are shown if they occurred in >10% in the imatinib group. Individual serious adverse events are shown if they occurred in ≥3 patients in either group.

(Circulation. 2013;127:1128-1138.)

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AV-101

Potential Targeted Treatment for PAH

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AV-101

AV-101 is a combination product comprised of a proprietary dry powder imatinib formulation in a capsule delivered by a dry powder inhaler designed to:

- Deliver drug to the lungs
- Limit systemic exposure
- Be easily administered

Imatinib molecular structure unmodified

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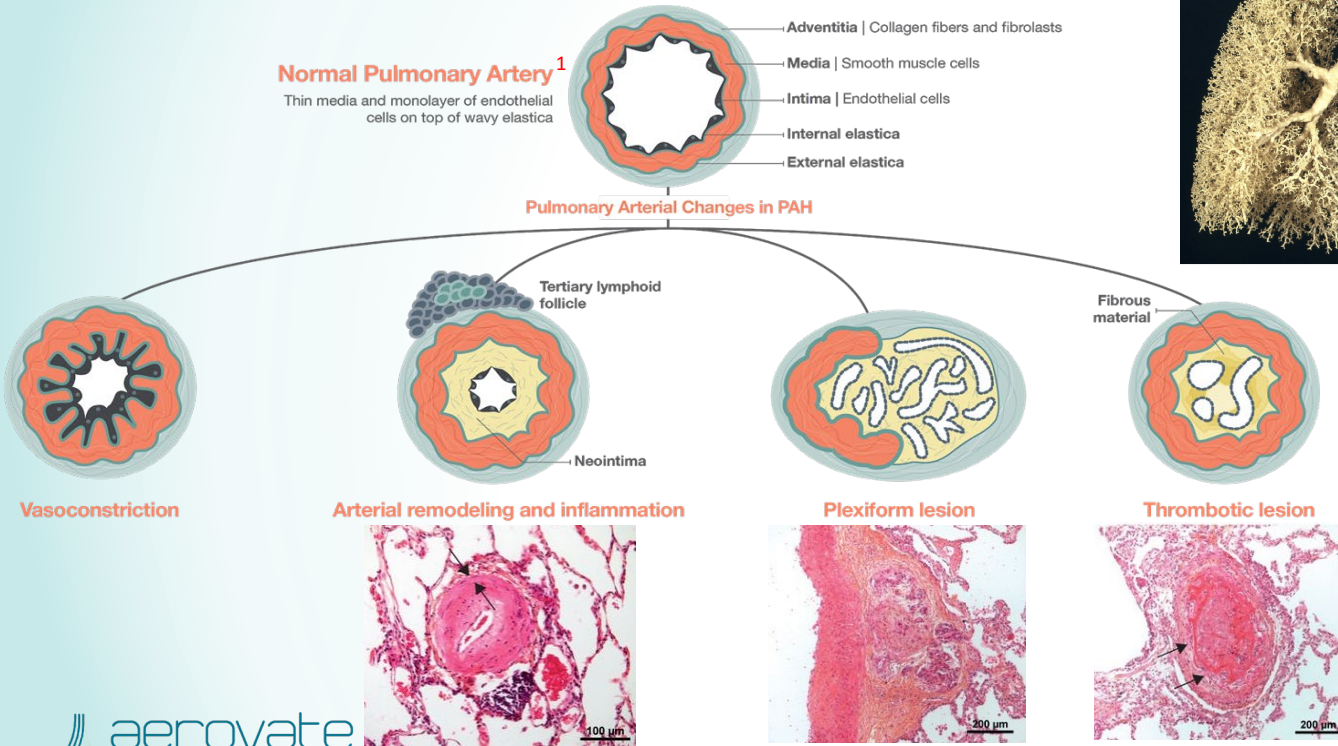
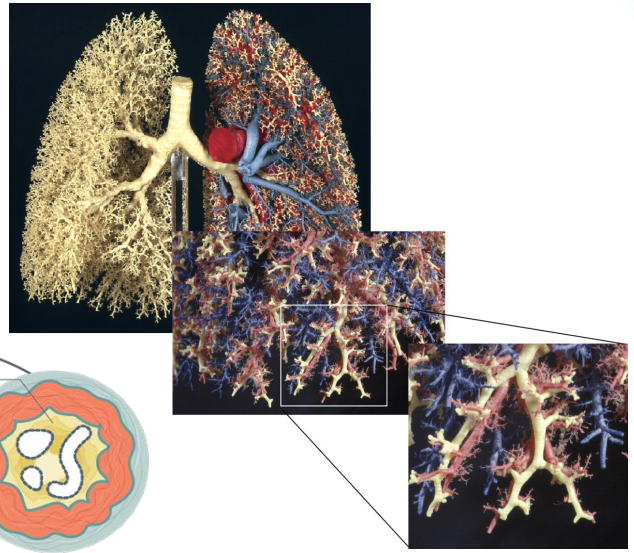
PAH is a Disease of All Layers of the Pulmonary Arteries





Latex cast of human airway (white), arterial tree (red), and venous tree (blue)[3]

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1. Adapted from J Am Coll Cardiol. 2015 May 12;65(18):1976-97. 2. Eur Respir J. 2019 Jan 24;53(1):1801887. 3. J Appl Physiol (1985). 2011 Apr;110(4):1119-29.)

AV-101 Phase 1 Trial

A single and multiple ascending dose Phase 1 study (n=82) in healthy volunteers to determine safety and tolerability

SAD	MAD	STUDY ASSESSMENTS
Five AV-101 cohorts from 1 mg to 90 mg **n=6 Active / 2 Placebo**	Seven days of BID dosing	Vital Signs
	Low dose 10 mg	Pulmonary Function Testing (FEV1 and FVC)
400 mg imatinib tablet for systemic exposure comparison **n=8**	Med dose 30 mg	Oxygen Saturations
	High dose 90 mg	QTc
		Adverse Events
n=8 per cohort	**n=12 per cohort; 9 Active / 3 Placebo**	

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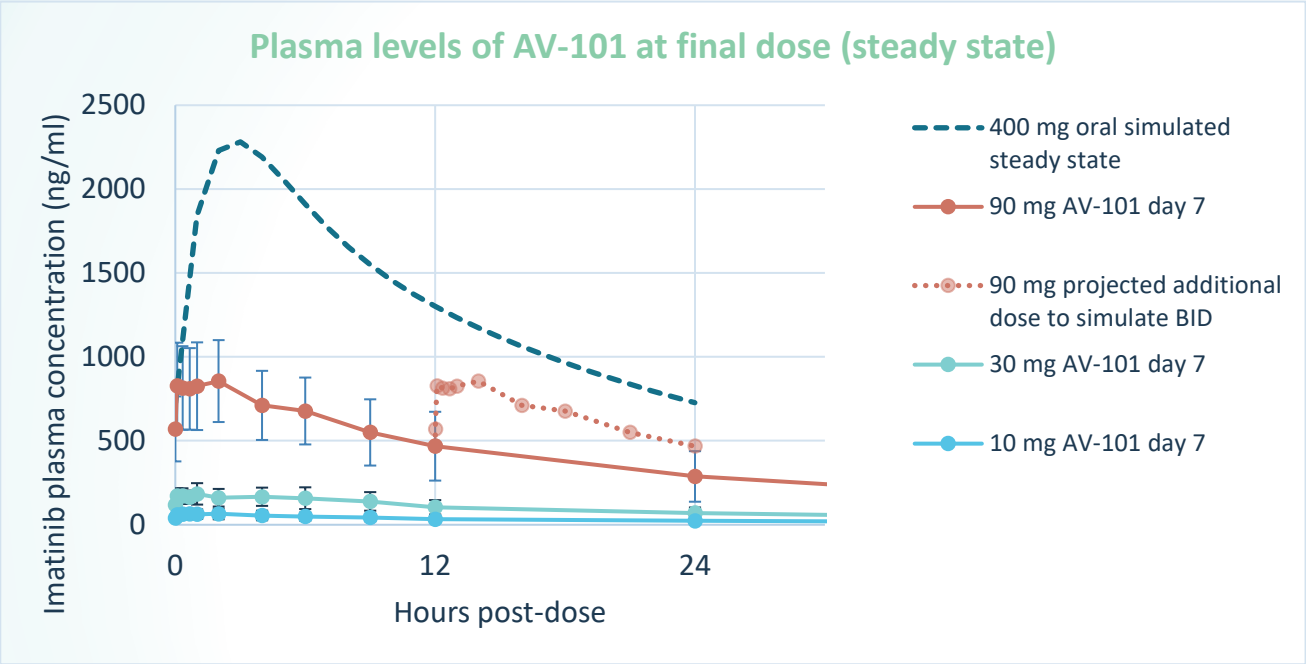
AV-101 Phase 1 Trial: Lower Systemic Exposures Observed



Systemic exposures for all doses of AV-101 observed to be lower than simulated steady-state exposure of 400 mg of oral imatinib

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AV-101 Phase 1 Tolerability Profile

AV-101 was generally well tolerated

- No serious adverse events were reported
- No change in pulmonary function, oxygen saturation, and QTc interval
- Most AEs mild to moderate
- One discontinuation due to vomiting on Day 1 in the MAD 90 mg Cohort
- Most common AE, cough, was at max dose and limited to within 30 minutes of dosing

Adverse Event n(%)	10 mg (n=8)	30 mg (n=9)	90 mg (n=9)
Cough	1 (13)	1 (11)	5 (56)
Persistent cough	-	-	-
Headache	-	-	4 (44)
Nausea	-	-	2 (22)
Chest discomfort	-	-	2 (22)
Throat irritation	-	1 (11)	1 (11)
Musculoskeletal pain	-	-	2 (22)

Single incidence AEs: Vomiting (discontinued), Dysgeusia, Musculoskeletal chest pain, Nasal congestion, Oropharyngeal pain, Back pain, Abdominal pain, Covid-19, Presyncope, Alanine aminotransferase increased

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Phase 2b Dose-Ranging Trial 2021

- Placebo controlled dose-ranging study
  - 10 mg, 35 mg and 70 mg AV-101 BID for 24 weeks
  - Targeting 200 patients across 4 dose groups
- Multicenter international trial
- Two or more background PAH therapies
- Functional class II - IV
- Primary endpoint: change in pulmonary vascular resistance (PVR) at 24 weeks
  - Statistically significant improvement on PVR already demonstrated with oral imatinib in multiple third-party clinical trials
  - Validated endpoint
  - Powered to detect statistically significant change
- Key secondary endpoints: Change in 6MWD, NTproBNP, QoL

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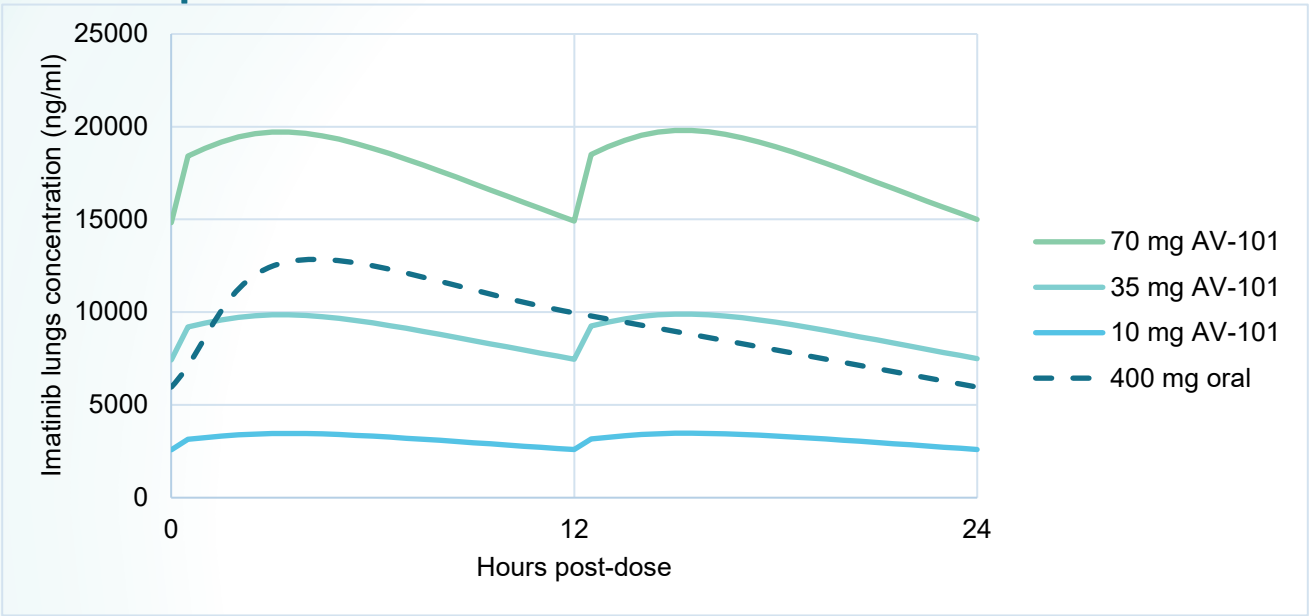
Expected AV-101 Lung Exposures of Planned Phase 2b Doses, Extrapolated based on Phase 1 Results



Predicted lung concentrations of planned Phase 2b doses of AV-101 overlap or surpass 400 mg oral imatinib dose. Predicted lung concentrations extrapolated based on a PK model using published method for the extrapolation of lung exposures, based on plasma levels observed in Phase 1 trial

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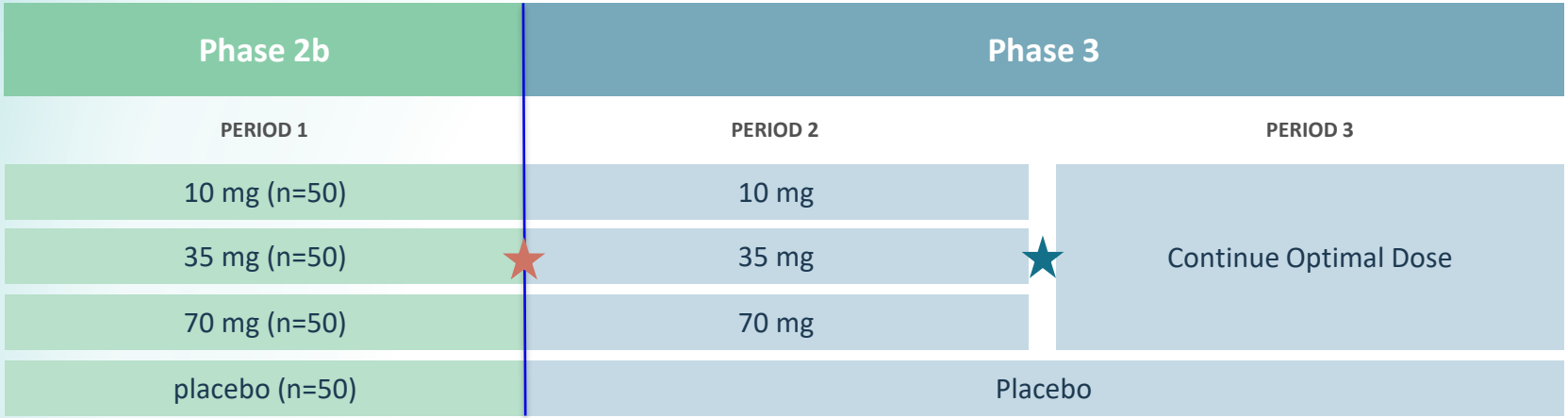
Phase 2b/3 Adaptive Continuous Trial of AV-101



- All patients are treated for 24 weeks
- 6MWD is primary endpoint in Phase 3

★ Phase 3 enrollment begins immediately upon completion of Phase 2b enrollment

★ Phase 3 dose selected based on Phase 2b data after 24 weeks of follow up

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PAH Clinical Pipeline For Novel Mechanisms is Limited

Potential PAH Therapies in Phase 3:

- Acceleron - Sotatercept (TGFβ ligand trap) in Phase 3

	Oral Imatinib Phase 3	Sotatercept Phase 2	
		Low	High
PVR	-31.8%	-20.5%	-33.9%
6MWD	+32 m p=0.002	+29 m p=0.02	+21 m p=0.08

The data in the chart above are based on a cross-trial comparison and not a head-to-head clinical trial. Such data may not be directly comparable due to differences in trial design, study protocols, conditions and patient populations.

Other Potential PAH Therapies in Development:

- Gossamer Bio - Seralutinib (inhaled TKI) in Phase 2
- Altavant Sciences - Rodatristat (tryptophan hydroxylase inhibitor) in Phase 2
- Pfizer - PF-06842874 (CDK4/6 inhibitor) in Phase 1

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AV-101: Unmet Need, Blockbuster Potential

Positioning

Added to 2 agents before prostacyclin

Added to 3 agents before parenteral prostacyclin

~50% (~17,500)

We estimate to be taking 2-3 drugs

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Positive Initial Regulatory Interactions and Feedback

Regulatory Interactions Completed

- FDA Pre-IND meeting in January 2020
- FDA and EMA Orphan Designation granted for AV-101 for the treatment of PAH
- Received EMA Scientific Advice in March 2021
- End-of-Phase-1 Meeting with FDA in April 2021

Feedback from FDA and EMA on Clinical Development

- A single Phase 2b/3 trial could support NDA
- Aligned on Phase 2b/3 endpoints for potential NDA/MAA submission

Intellectual Property Protection Estimated to 2040+

AV-101 is a combination product candidate uniquely built as an aerosol

- Patent applications directed to the composition of the aerosol formulation
- Barriers-to-entry against competitors
- Patent applications directed to the aerosol, drug product, manufacturing and methods of treatment

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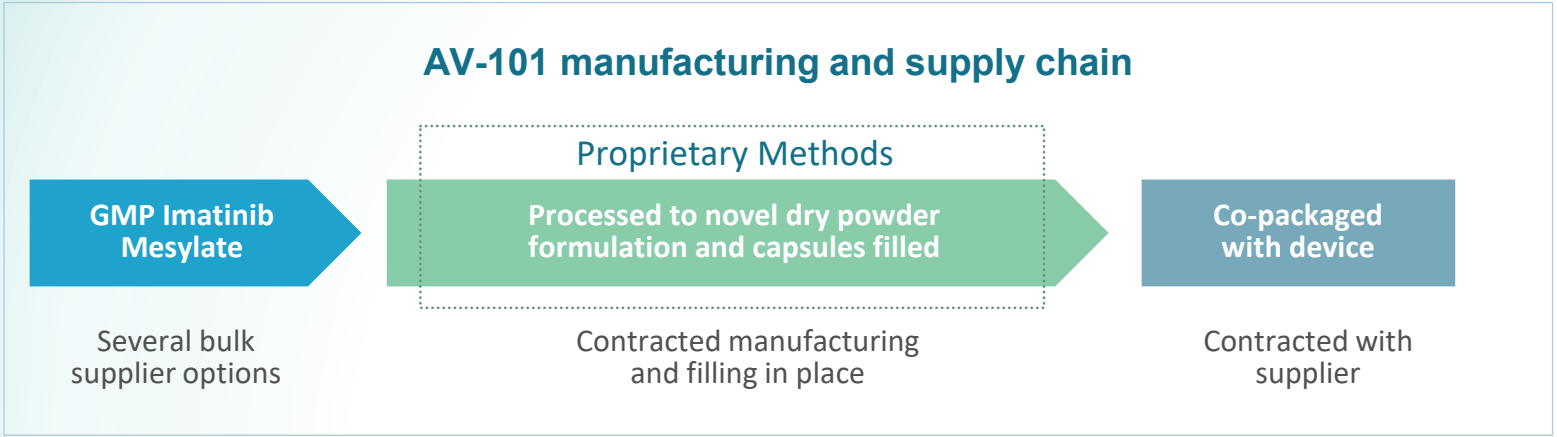
Chemistry, Manufacturing and Controls



Stability of AV-101

- API stability tested through 18 months
- Bulk aerosol powder stability through 12 months
- Filled product stability testing ongoing

AV-101 Device and Delivery Performance

AV-101 Device

- Off-the-shelf commercial scale dry powder inhaler
  - No batteries, compressors or cords
  - No sterile vials containing solutions or suspensions
  - CE mark in EU and Device Master File registered with FDA
- Designed for ease of use and convenience
- Intended dosing of 2 capsules twice a day
- Replaced weekly

AV-101 Delivery Performance

- Consistently high delivered and fine particle dose
- Ideal size and size distribution for lung penetration

NOTE: Device planned for Phase 2b/3 trial was not used in Phase 1 trial, but delivery performance has been assessed.

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Thank you!