



Freedom from a lifetime of disease

Corporate Presentation

September 2019

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potential regulatory approvals and the timing thereof, anticipated benefits of our gene therapy platform, the expected safety profile of our investigational gene therapies, timing and likelihood of success, plans and objectives of management for future operations, future results of anticipated products, and the market opportunity for our investigational gene therapies. Any such statements in this presentation that are not statements of historical fact may be deemed to be forward-looking statements.

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Developing gene therapies designed to cure rare diseases

- + Deep pipeline targeting lysosomal storage disorders (LSDs) where SoC ~\$4B 2018 net sales
- + Compelling Fabry data across Phase 1 and Phase 2 trials
- + Gaucher and cystinosis expected to enter the clinic this year
- + Powered by plato™ - our commercial-stage manufacturing platform
- + Management comprised of cell, gene and rare disease industry leaders
- + Multiple 2H 2019 milestones anticipated

Cell, gene and rare disease industry leaders



MANAGEMENT TEAM



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President and CEO



Birgitte Volck, PhD, MD
President of Research and Development



Kim Warren, PhD
Head of Operations



Erik Ostrowski
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Chris Mason, MD,
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Steady stream of clinical programs

4 clinical trials up and running













Investigational Gene Therapy		Proof-of-Concept	IND-Enabling	Phase 1/2	Commercial Rights
Fabry AVR-RD-01		<div><div>Phase 1</div><div>Phase 2</div></div>			AVROBIO
Gaucher AVR-RD-02		<div>Phase 1/2</div>			AVROBIO
Cystinosis AVR-RD-04		<div>Phase 1/2</div>			AVROBIO
Pompe AVR-RD-03		<div>Pre-Clinical</div>			AVROBIO

Addressing multi-billion dollar markets



CURRENT STANDARD OF CARE COSTS

Disease	Est. Cost Per Year	Approx. 2018 Net Sales	Selected Companies
<i>Fabry</i>	\$320k	\$1.4B	SANOFI GENZYME   
<i>Gaucher</i>	\$250k-400k	\$1.4B	SANOFI GENZYME   
<i>Pompe</i>	\$500k	\$1B	SANOFI GENZYME 
<i>Cystinosis</i>	\$625k-700k*	\$0.2B	  

Sources: Rombach S et al, Orphanet J Rare Dis, 2013; van Dussen L et al, Orphanet J Rare Dis, 2014;

WAC pricing from Redbook; 2018 Net Sales from company annual and other reports

*= for Horizon's Procysbi oral therapy (delayed release cysteamine bitartrate)

Note: Shire acquired by Takeda in 2019

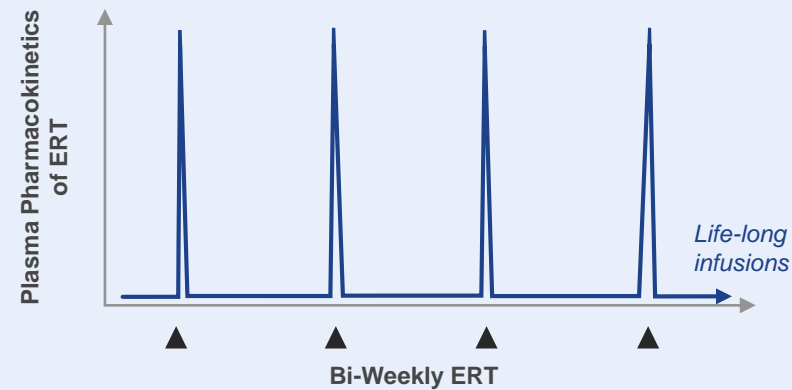
Life-long treatments vs. potential single dose cure



DISEASE PROGRESSION CONTINUES

Enzyme Replacement Therapy (ERT)

Temporary bolus of enzyme, not curative



Enzyme or protein level

Transient, intermittent elevation

Treatment burden

Bi-weekly IV infusions

DISEASE PROGRESSION COULD HALT

AVROBIO Gene Therapy

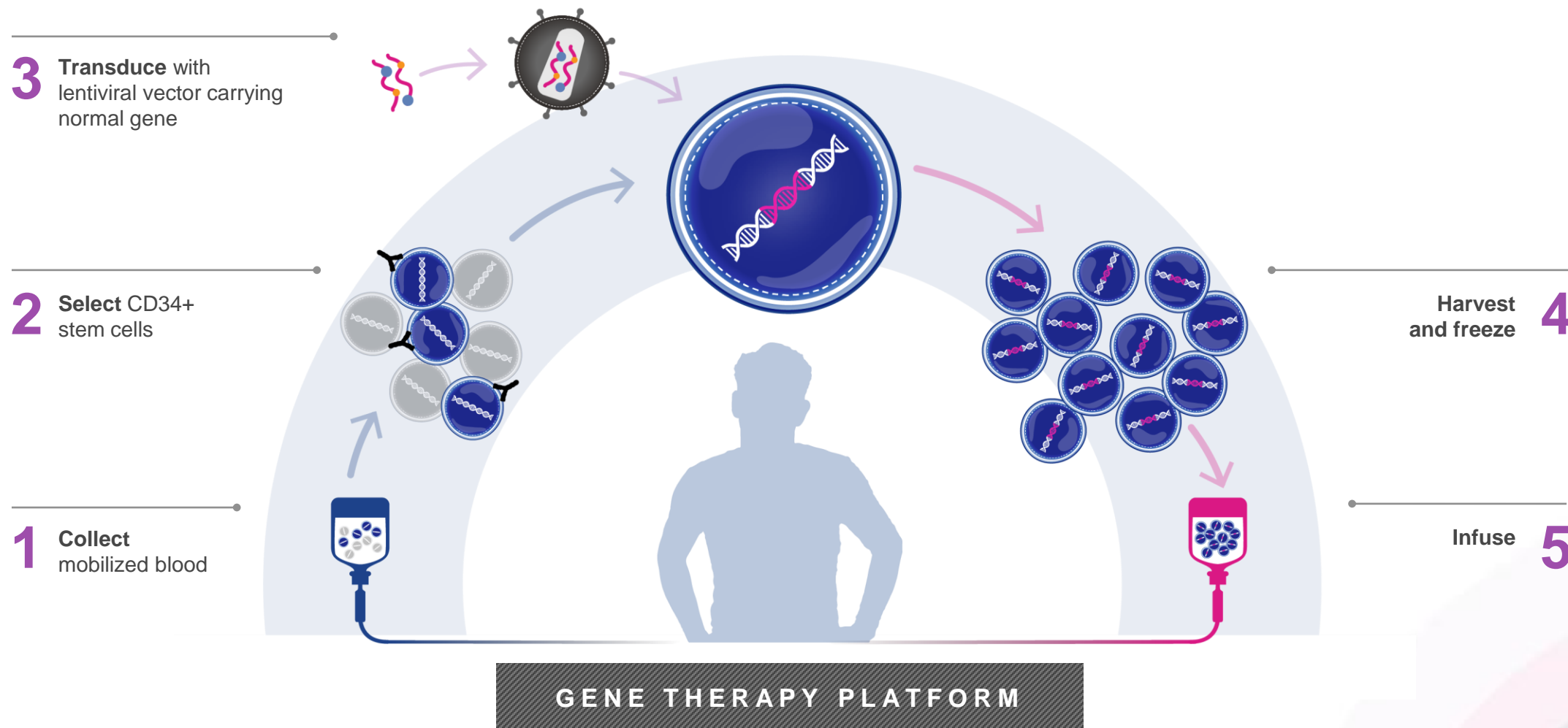
24/7 expression of protein, curative potential



Long-term, continuous elevation

Single IV infusion

One platform applied across our portfolio



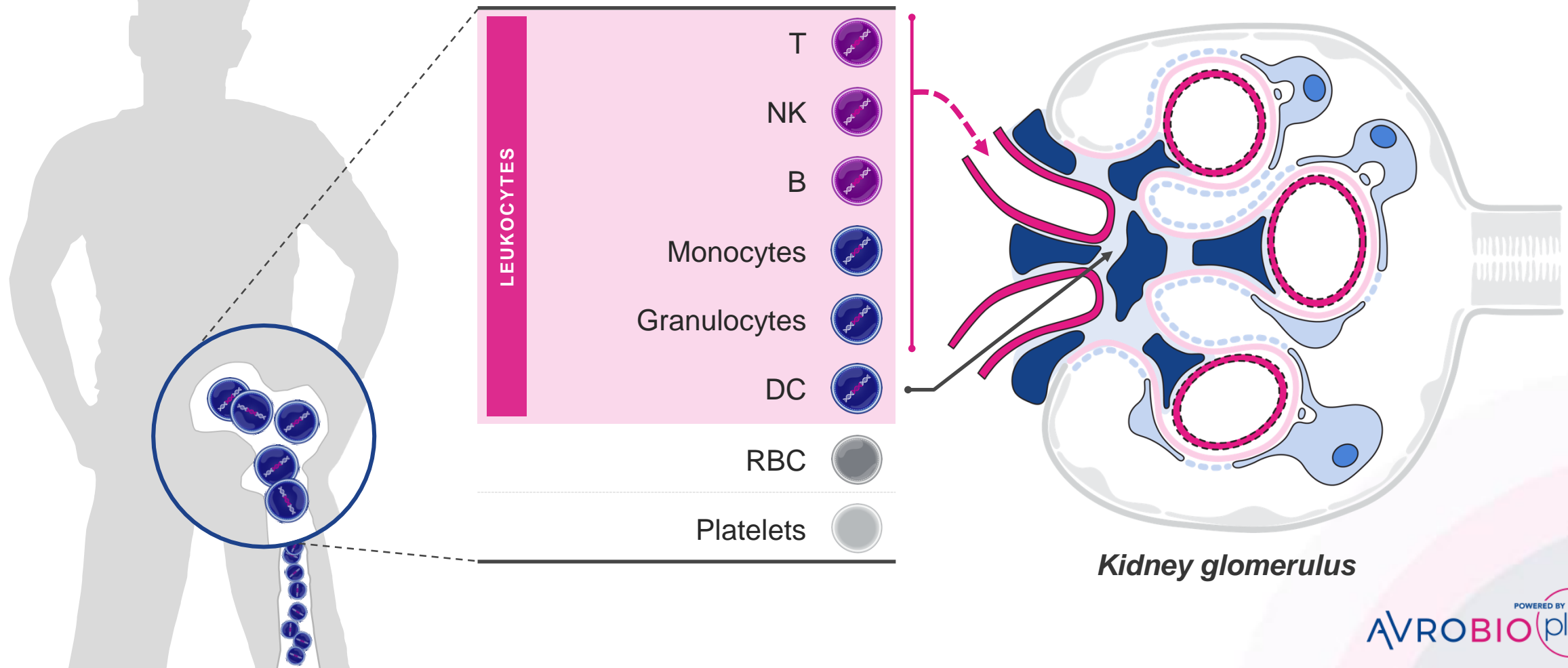
Endogenous enzyme delivered to tissues via multiple cell lineages



Long-term engraftment
in bone marrow

Manufacturing, transportation
and delivery in blood

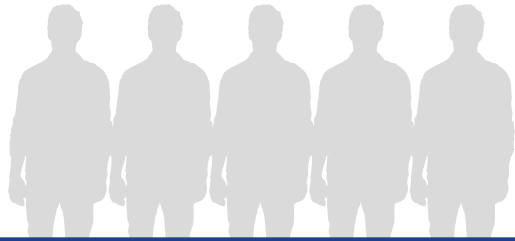
Example target organ





Two AVR-RD-01 Fabry clinical trials

8 patients dosed across Phases 1 and 2



PHASE 1

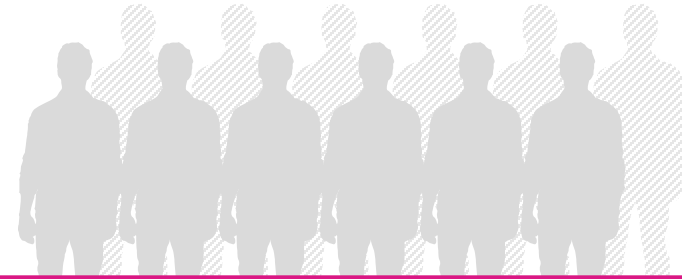
Investigator-Sponsored Trial*

Patients

n = 5 (fully enrolled)
On ERT > 6 months prior to enrollment
18 - 50 year-old males

Key Objective

Safety and preliminary efficacy



PHASE 2

AVRO – FAB-201 Trial

Patients

n = 8-12 (3 patients dosed to-date)
Treatment-naïve
16 - 50 year-old males

Key Objectives

Safety and efficacy

* Sponsored by FACTs team (Fabry Disease Clinical Research and Therapeutics) in Canada

FAB-201 Primary and secondary endpoints



FAB-201 Primary efficacy endpoint

Average number of Gb3 inclusions per kidney peritubular capillary (PTC)

- Biopsy at 1 year vs. baseline
- FDA-recognized endpoint in Fabry

Primary safety endpoints



AEs, SAEs
Clinical labs, ECG, vital signs
Antibodies, RCL, ISA

Secondary efficacy endpoints



ORGAN AND SYSTEM FUNCTION

Kidney function
Cardiac function
GI distress
Pain



PATIENT WELL-BEING

Clinical status
Quality of life



BIOMARKERS

Toxic metabolite – lyso-Gb3 in plasma, urine
Substrate – Gb3 in plasma, urine, skin
Enzyme – AGA in leukocytes, plasma
VCN



Gb3, also referred to as GL-3: a type of fat that builds in cells, resulting in damage to kidneys, heart and brain

Peritubular capillaries (PTCs), also referred to as kidney interstitial capillaries (KICs) convey blood after filtration in the glomeruli, enabling it to eventually exit the kidneys and return to the circulatory system



FAB-201 Patient Characteristics

	PATIENT 1	PATIENT 2	PATIENT 3
Age symptom onset / diagnosis	10 / 19 years	36 / 37 years	13 / 13 years
Age dosed with AVR-RD-01	21 years	46 years	40 years
Mutation	c.1021G>A (p.E341K)	c.644A>G (p.N215S)	c.639+1G>T
Primary disease signs and symptoms	<ul style="list-style-type: none">• Kidney disease• Chronic pain• GI symptoms• Decreased cold sensation	<ul style="list-style-type: none">• Cardiac disease• Peripheral neuropathy• Chronic pain• Increased tiredness• GI symptoms• Intermittent tinnitus• Mild high frequency hearing loss• Raynaud's syndrome	<ul style="list-style-type: none">• Kidney disease• GI symptoms• Peripheral neuropathy• Bilateral deafness• Tinnitus• Peripheral edema• Decreased cold sensation
Leukocyte AGA enzyme activity at baseline (nmol/h/mg)	0.10*	2.38**	0.58**
Plasma lyso-Gb3 at baseline (nM)**	202	8	147
Comment	IgA deposits in kidney biopsy	Cardiac variant, not a classic Fabry male	

* Mayo Lab, ref range ≥ 23.1 nmol/h/mg

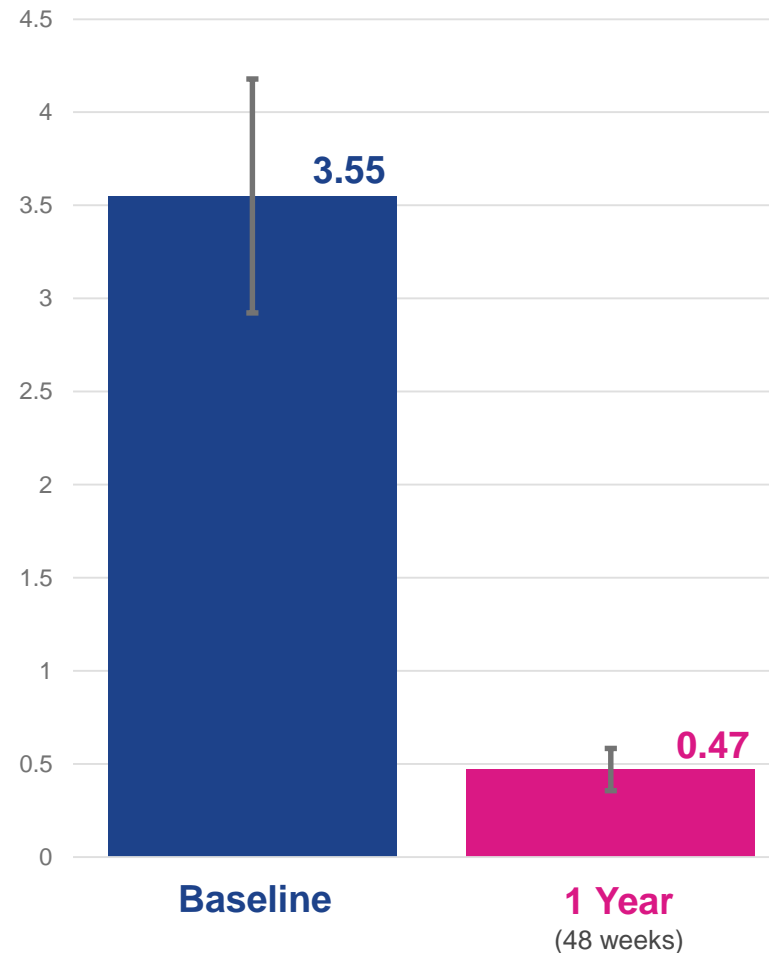
** Rupa Lab, ref range 24-56 nmol/h/mg

*** Reference value ≤ 2.4 nM



FAB-201 Patient 1: 87% substrate reduction in kidney biopsy

Average number of **Gb3** inclusions per peritubular capillary (PTC)



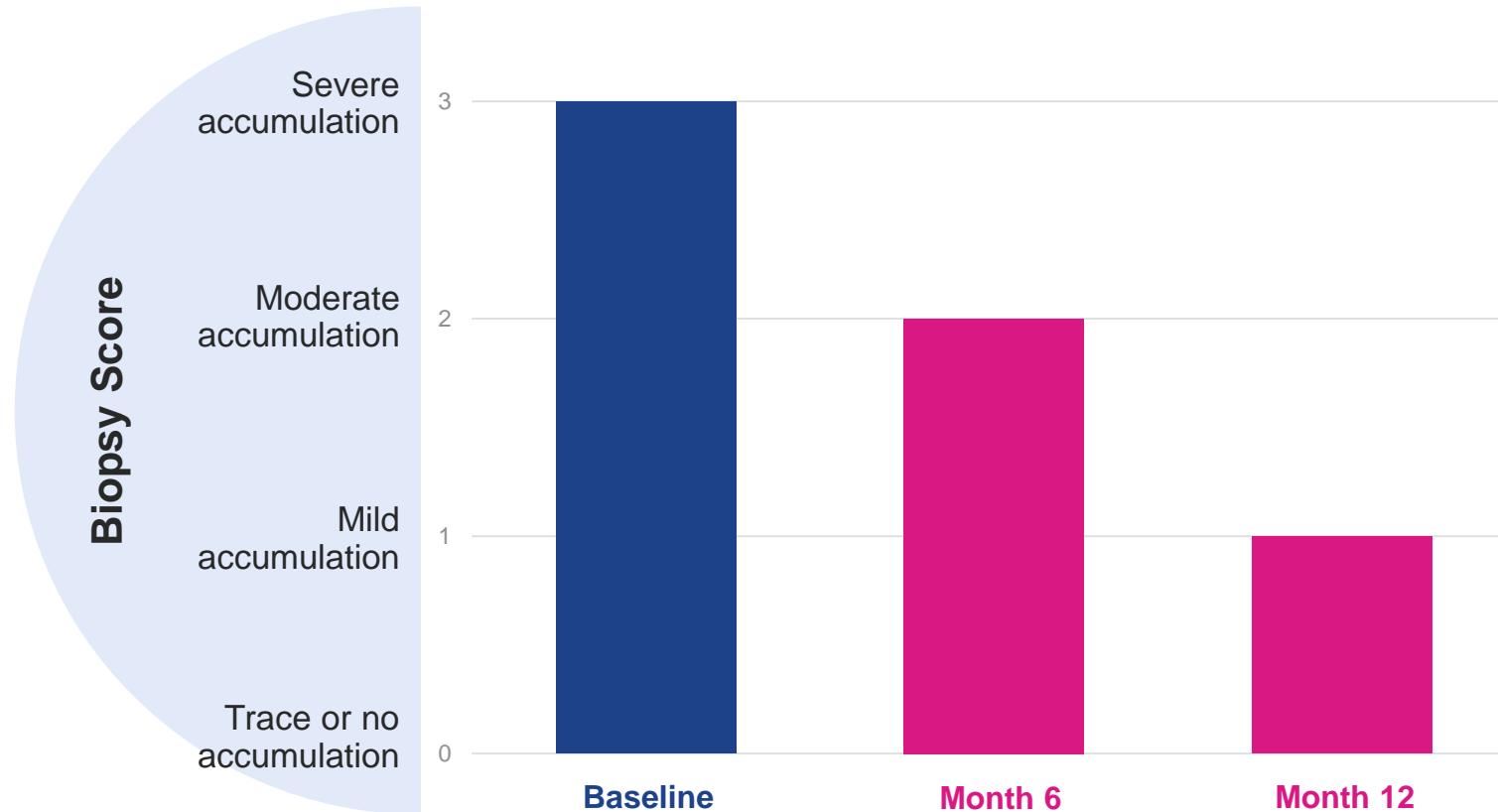
- Unpaired *t* test for difference between *n*=55 PTCs at baseline vs. *n*=101 PTCs at 1 year; *p* < 0.0001
- Error bar represents the standard deviation

Baseline: The last available, non-missing observation prior to AVR-RD-01 infusion

Note: With respect to Fabry disease, Gb3 inclusions per PTC is interchangeable with GL-3 inclusions per KIC

FAB-201-1: First patient in FAB-201 clinical trial

FAB-201 Patient 1: Continued reduction in substrate inclusions in skin endothelial cells

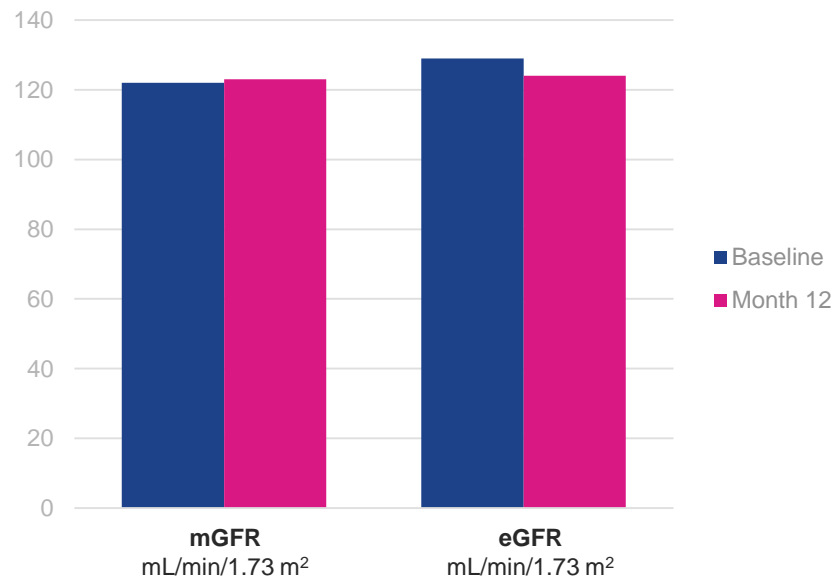




FAB-201 Patient 1: Kidney and cardiac function stable at one year



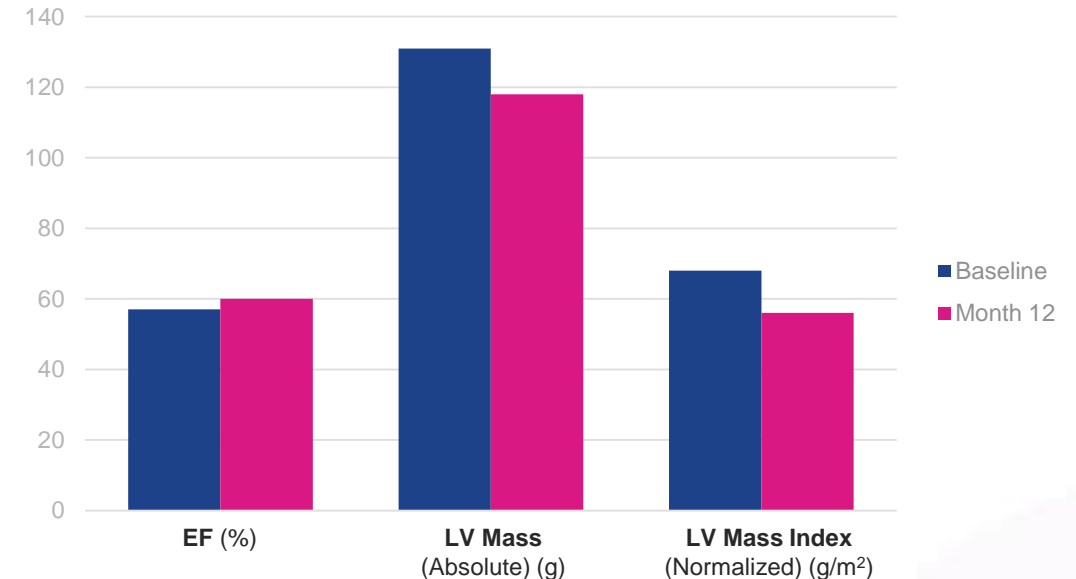
KIDNEY FUNCTION
remains within normal range



Normal Range	mGFR/eGFR
Male (20-29 years)	Average 116*



CARDIAC FUNCTION
remains within normal range



Reference Range Mean Values ± SD	EF (%)	LVM (g)	LVMI (g/m²)
Male (20-39 years)	64.3 ± 4.2	138.9 ± 24.5	67.8 ± 10.7

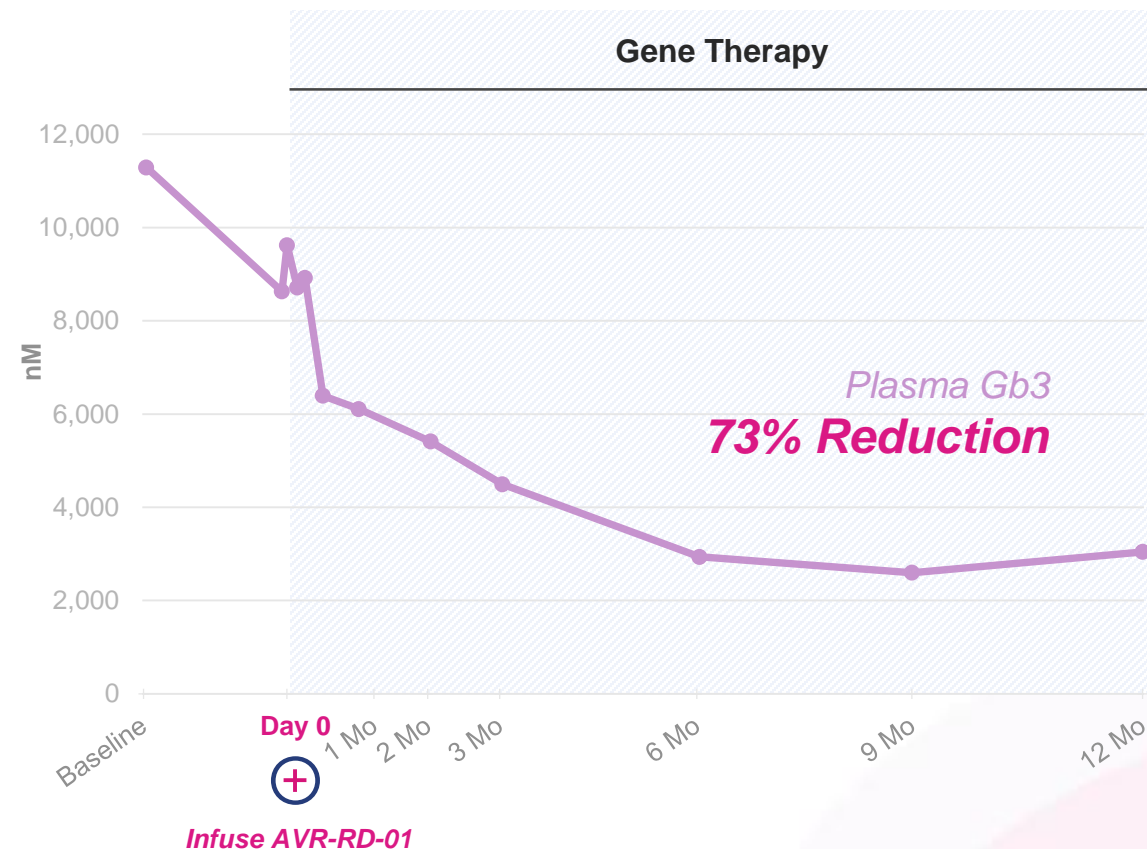
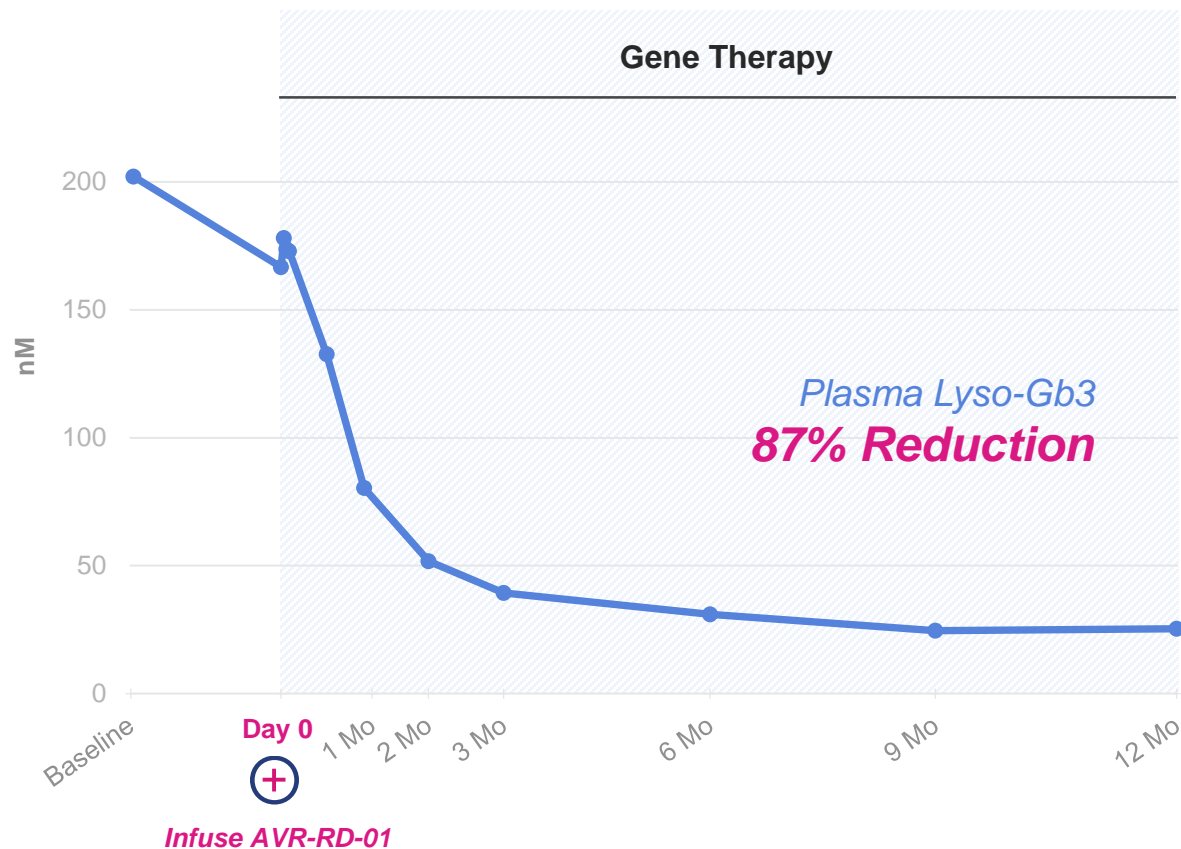
Source: <https://www.kidney.org/atoz/content/gfr>

Note: mGFR is measured Glomerular Filtration Rate, eGFR is estimated Glomerular Filtration Rate

Source: Alfakih K et al, J Magn Reson Imaging, 2003

Note: EF is Ejection Fraction, LVMI is Left Ventricular Mass Index

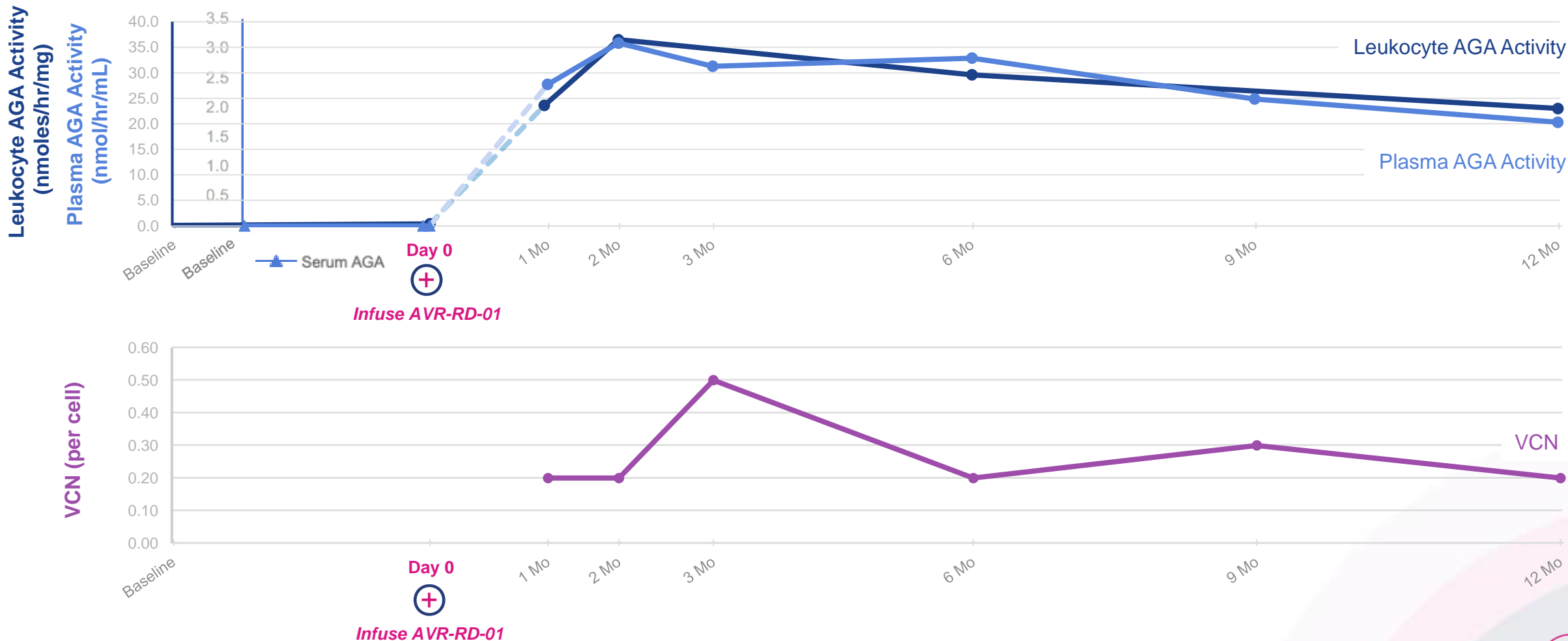
FAB-201 Patient 1: Substantial reduction in plasma substrate / metabolite levels, sustained at 1 year



Baseline: The last available, non-missing observation prior to AVR-RD-01 infusion

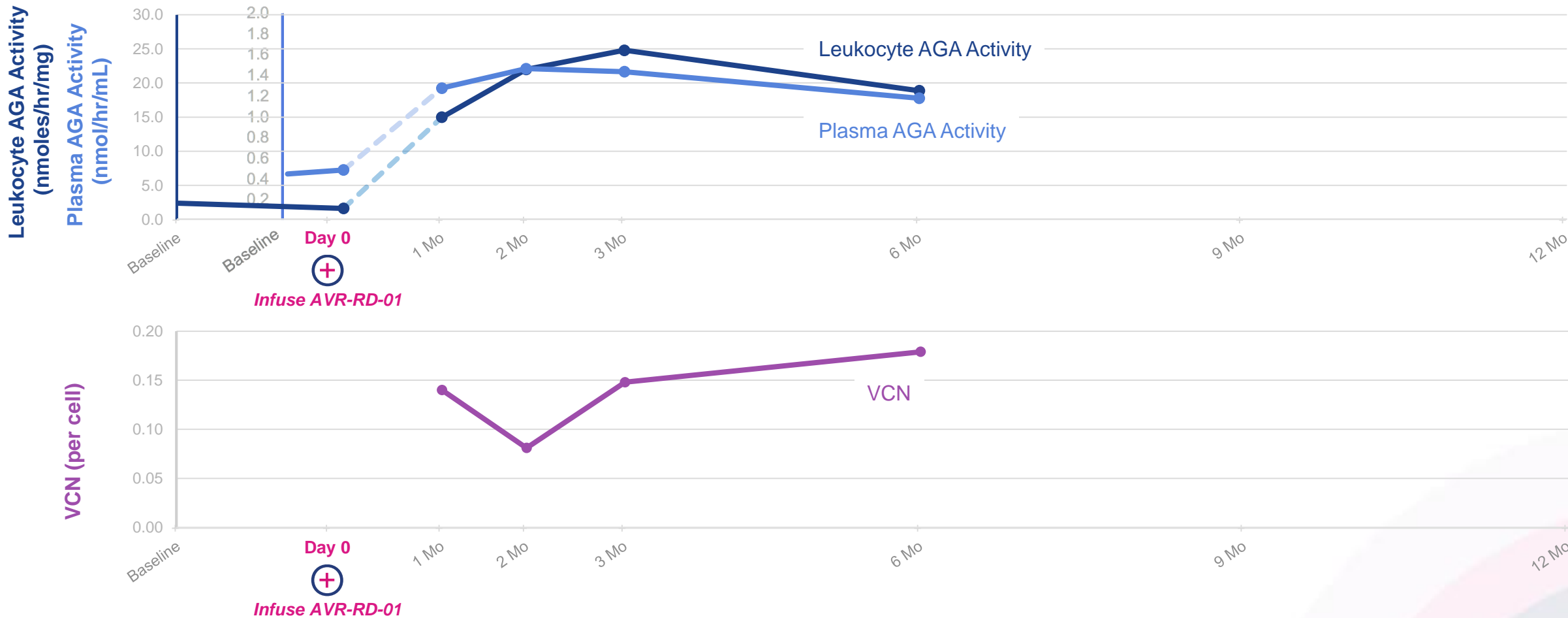
Note: AVR-RD-01 is an investigational gene therapy

FAB-201 Patient 1: Sustained leukocyte and plasma enzyme activity at 1 year; VCN stable



Note: 0.1 VCN is indicative of approx. 5-10% of all nucleated cells having an average of 1-2 copies of the transgene
Baseline: The last available, non-missing observation prior to AVR-RD-01 infusion

FAB-201 Patient 2: Sustained leukocyte and plasma enzyme activity and VCN at 6 months



Note: Patient 3 had plasma AGA activity of 0.740, leukocyte AGA activity of 9.94 and VCN of 0.12 as of 1 month

Note: 0.1 VCN is indicative of approx. 5-10% of all nucleated cells having an average of 1-2 copies of the transgene

Baseline: The last available, non-missing observation prior to AVR-RD-01 infusion



FAB-201
3 patients dosed

**No unexpected
trends or safety
events identified**



No AEs or SAEs related to AVR-RD-01 drug product



AEs and SAEs reported

- **AEs**
 - Generally consistent with myeloablative conditioning, underlying disease or pre-existing conditions
- **SAEs**
 - **Pre-treatment**
 - Seizure (resolved)
 - **Post-treatment**
 - Dehydration, nausea, vomiting (resolved)
 - Febrile neutropenia (resolved)



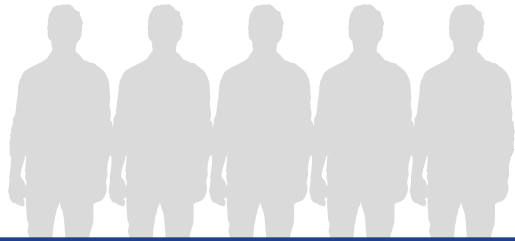
Anti-AGA antibodies

- Transient low titer in 1 subject (resolved)



Two AVR-RD-01 Fabry clinical trials

8 patients dosed across Phases 1 and 2



PHASE 1

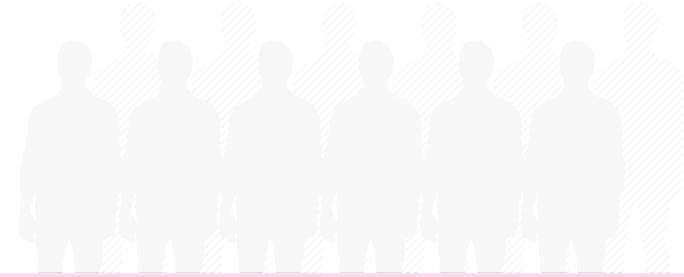
Investigator-Sponsored Trial*

Patients

n = 5 (fully enrolled)
On ERT > 6 months prior to enrollment
18 - 50 year-old males

Key Objective

Safety and preliminary efficacy



PHASE 2

AVRO – FAB-201 Trial

Patients

n = 8-12 (3 patients dosed to-date)
Treatment-naïve
16 - 50 year-old males

Key Objectives

Safety and efficacy

* Sponsored by FACTs team (Fabry Disease Clinical Research and Therapeutics) in Canada



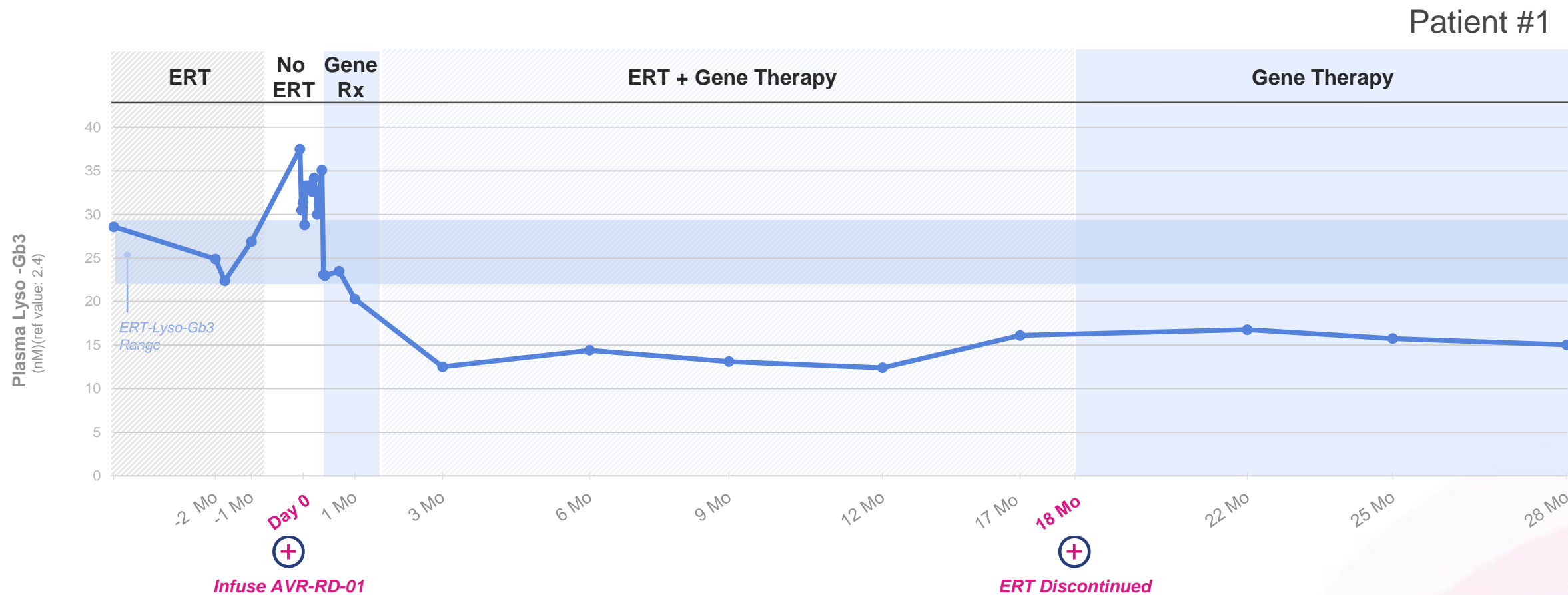
Phase 1 Patient Characteristics

	PATIENT 1	PATIENT 2	PATIENT 3	PATIENT 4	PATIENT 5
Age symptom onset / diagnosis	18 / 37	9 / 29	10 / 0	7 / 4	10 / 14
Years on ERT	11	6	4	11	2
Age dosed with AVR-RD-01	48	39	40	37	30
Mutation	c.962A>G (p.Q321R)	c.1033T>C (p.S345P)	c.427G>C (p.A143P)	c.427G>C (p.A143P)	(p.Y134S)
Primary disease signs and symptoms	<ul style="list-style-type: none">• Kidney disease• Cardiac disease• GI pain• GI diarrhea• Angiokeratoma• Insomnia	<ul style="list-style-type: none">• Kidney disease• Cardiomyopathy• Hypohidrosis• Corneal verticillata• Peripheral neuropathy• GI symptoms• Angiokeratoma• Lymphedema• Acroparesthesia	<ul style="list-style-type: none">• Cardiac Disease• Tinnitus• Headaches• Dizziness• Acroparesthesia	<ul style="list-style-type: none">• Cardiac Disease• Hypohidrosis• Tinnitus• Corneal verticillata• Angiokeratoma• GI symptoms	<ul style="list-style-type: none">• Kidney disease• Hypertension• Hypohidrosis• Tinnitus• Migraines• Impaired hearing• Angiokeratoma• Sleep apnea• Asthma• Depression
Leukocyte AGA activity at baseline* (nmol/h/mg)	2.1	1.1	0.6	2.2	1.0
Plasma lyso-Gb3 at baseline (nM)**	25	26	59	29	16
Discontinued ERT	18 months after gene therapy dose		Did not resume ERT after gene therapy dose	7 months after gene therapy dose	

* Rutar Lab, ref range 24-56 nmol/h/mg
** Reference value ≤ 2.4 nM

Phase 1: Plasma lyso-Gb3 reduction sustained >2 yrs

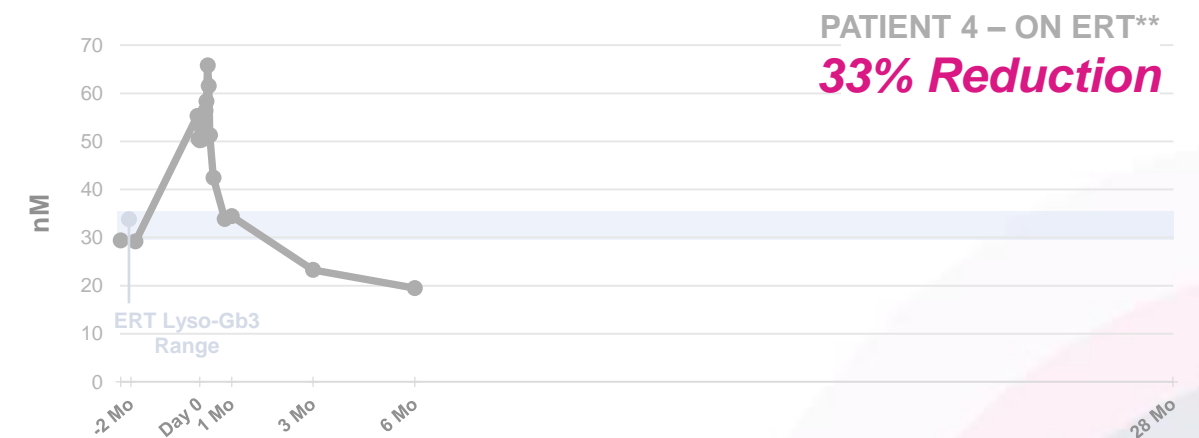
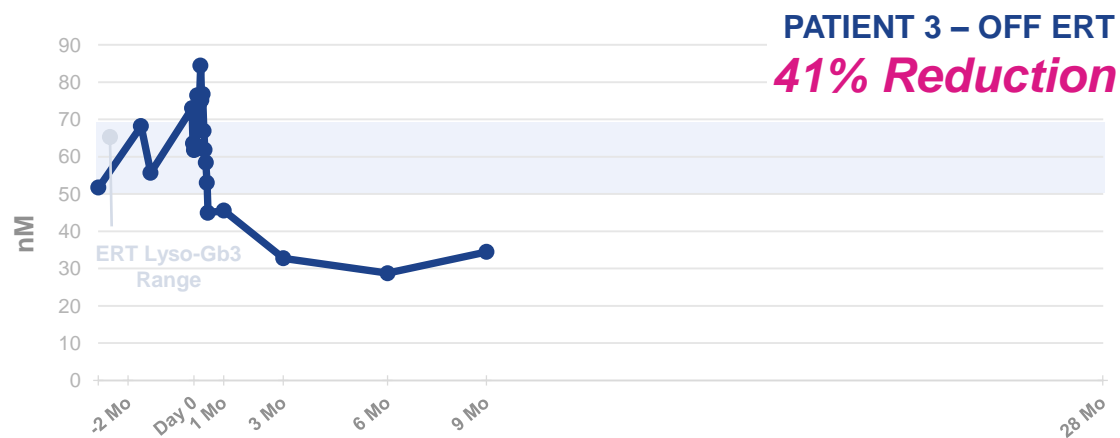
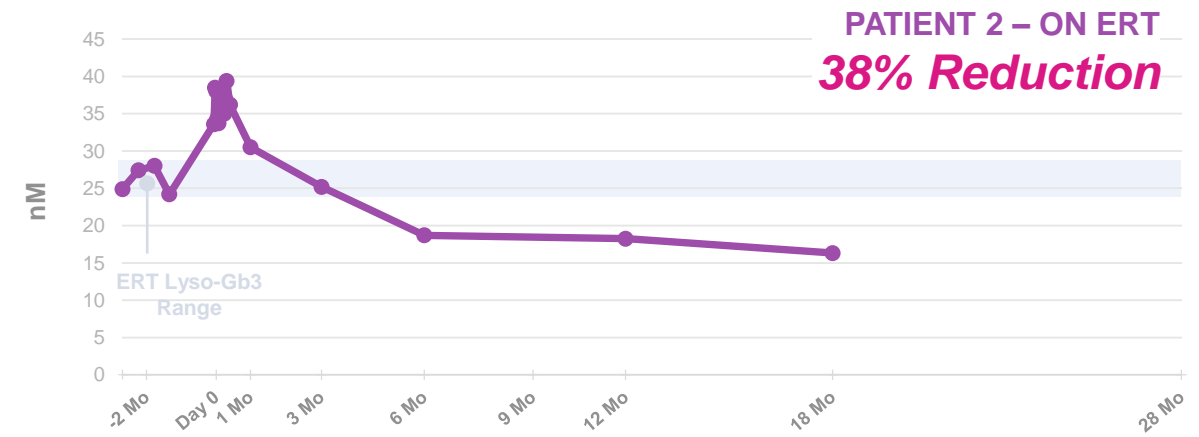
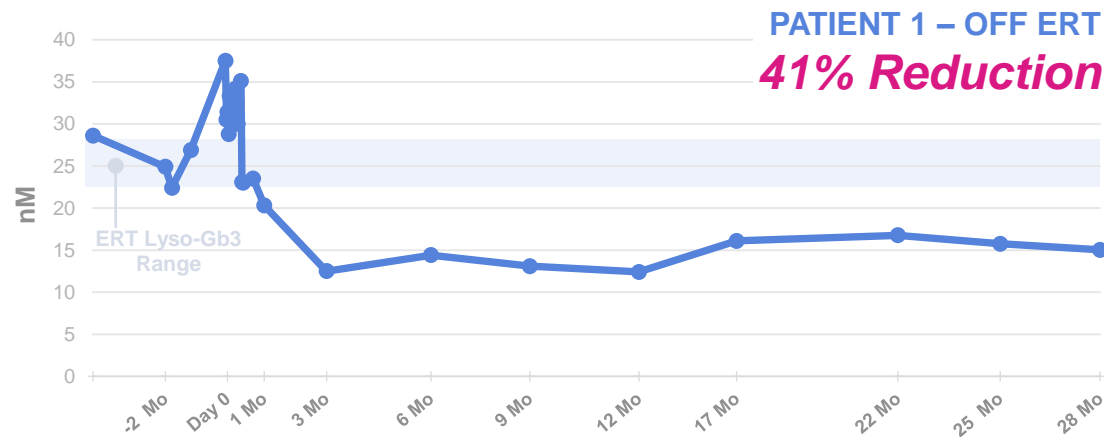
Reduced 41% from ERT baseline*



*Baseline: The mean of the values reported prior to initiating mobilization

Note: AVR-RD-01 is an investigational gene therapy candidate

Phase 1: Plasma lyso-Gb3 consistently reduced by 33-41% vs. baseline* ERT at 6+ months post AVR-RD-01 treatment



*Baseline: The mean of the values reported prior to initiating mobilization

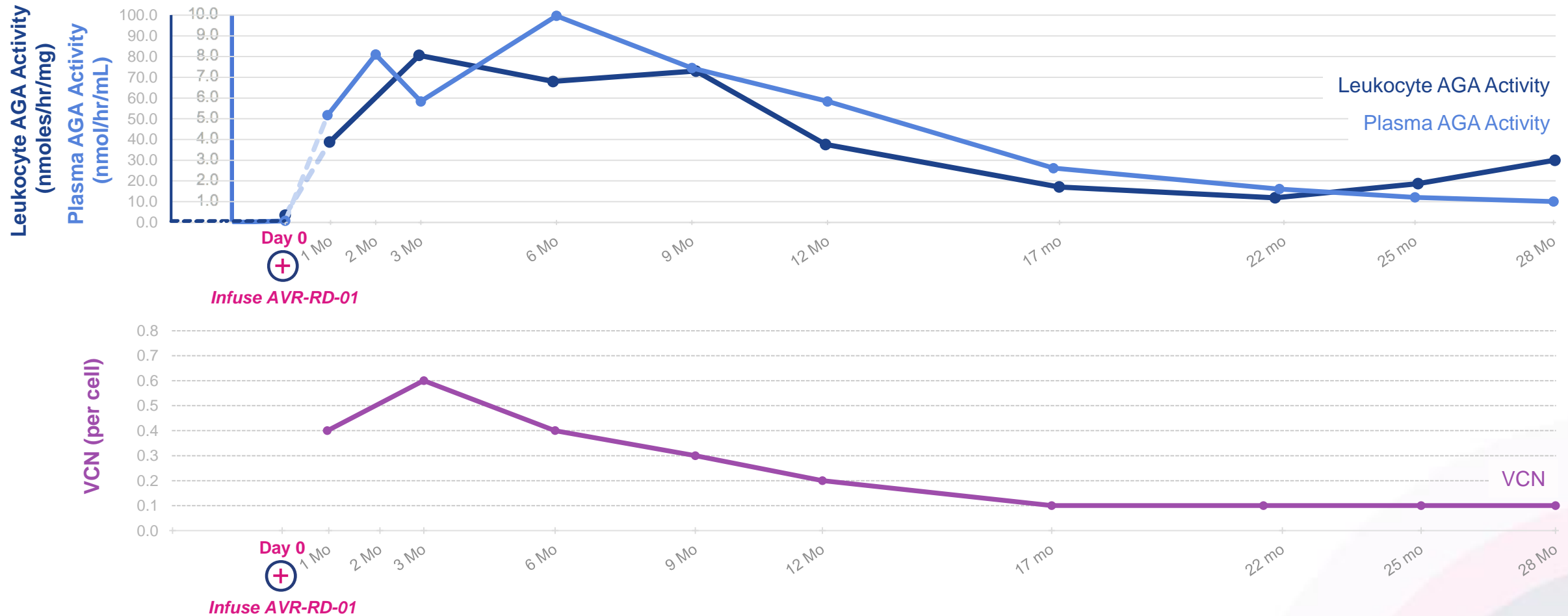
Percent reduction: As measured from baseline to last assessment

**Patient 4 discontinued ERT 7 months after gene therapy dose

Phase 1: Leukocyte and plasma enzyme activity sustained >2 years; VCN stable

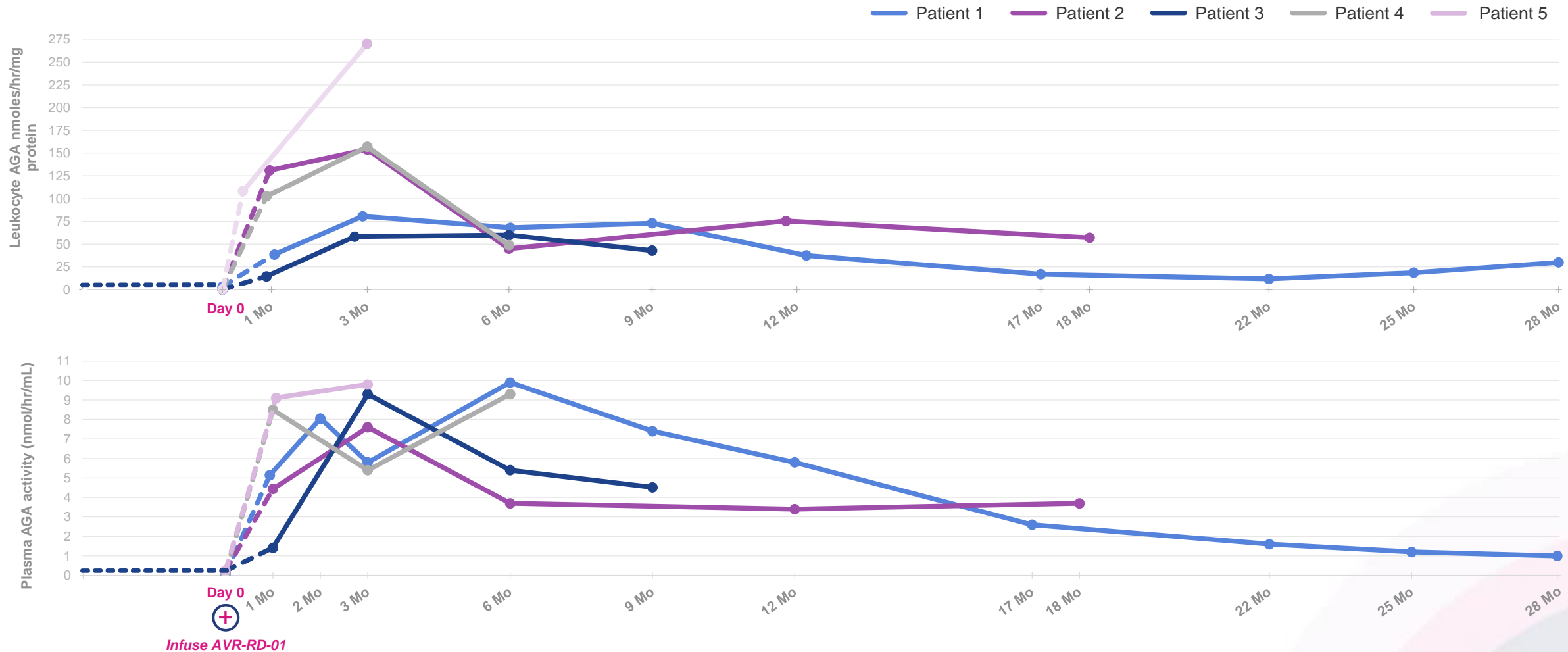


Patient #1



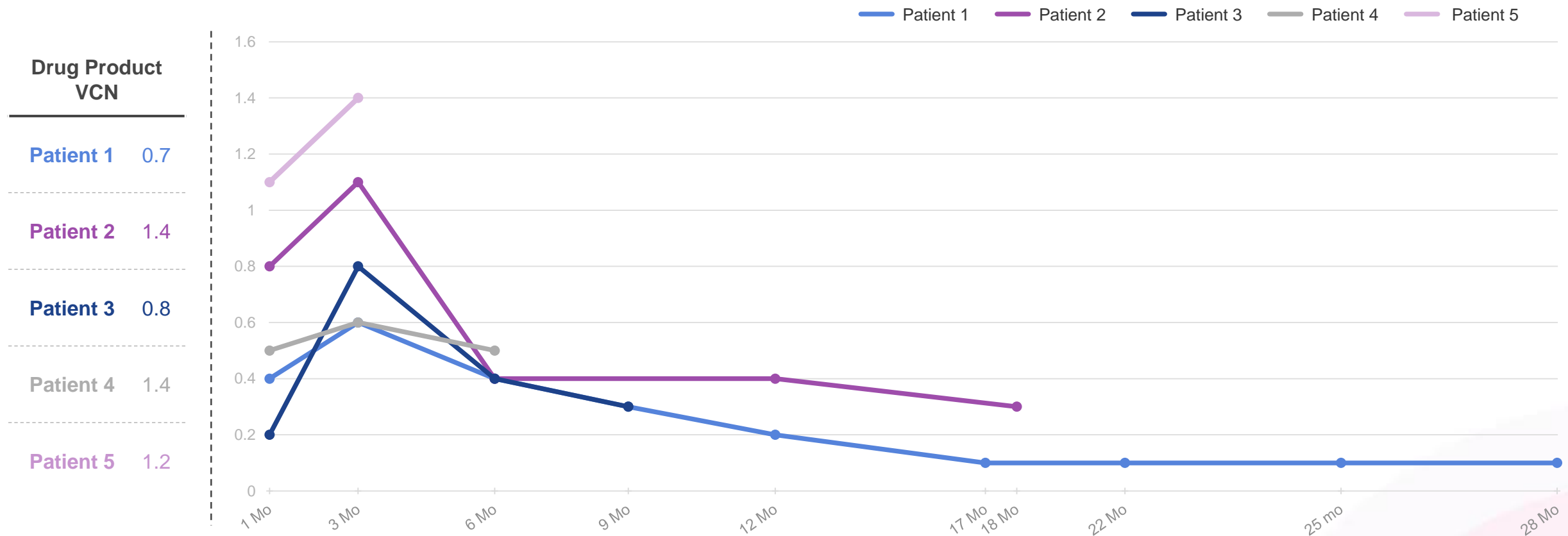
Note: 0.1 VCN is indicative of approx. 5-10% of all nucleated cells having an average of 1-2 copies of the transgene

Phase 1: Leukocyte and plasma enzyme activity levels trend consistently across all patients



Note: Enzyme measurements are taken at ERT troughs; Note: Dotted line illustrative only
Patient #5's Day 12 data point was utilized since the one month data was not obtained

Phase 1: Consistent VCN trend across all patients



Note: 0.1 VCN is indicative of approx. 5-10% of all nucleated cells having an average of 1-2 copies of the transgene



Phase 1
5 patients dosed

**No unexpected
trends or safety
events identified**



No SAEs related to AVR-RD-01 drug product



AEs and SAEs reported

- **AEs**
 - Generally consistent with myeloablative conditioning, underlying disease or pre-existing conditions
- **SAEs**
 - Febrile neutropenia (resolved)
 - Thrombophlebitis (resolved)*



Anti-AGA antibodies

- Mild titer rise in 1 patient






Note: Safety database cut as of May 24, 2019

**Resolved post-safety database cut-off date*

8 patients
dosed across
2 trials

longest follow-up
>2 years

Emerging data support potential first-line use in Fabry disease

-  87% decrease in Gb3 in first kidney biopsy at 1 year
in first Phase 2 patient
-  Plasma lyso-Gb3 reduced by 30-40% vs. baseline ERT
in four Phase 1 patients
-  Kidney and cardiac function stable at 1 year
in first Phase 2 patient
-  Durability sustained >2 years for enzyme activity and VCN
in first Phase 1 patient
-  No unexpected trends or safety events identified
8 patients across 2 trials

GAU-201: Phase 1/2 study in Gaucher Type 1 patients



GAUCHER
DISEASE
TYPE 1
ERT-STABLE and
TREATMENT NAÏVE
PATIENTS



Day -60

Mobilize stem cells

Patients on ERT:
ERT discontinued D -14

Day -4

Conditioning

Day 0

Infuse
AVR-RD-02

Day 28

Post-treatment
assessment

Months

3, 6, 9 & 12

Safety & efficacy
assessments

An adaptive, open-label, multinational phase 1/2 study of the safety and efficacy of ex vivo, lentiviral vector mediated gene therapy AVR-RD-02 for patients with Type 1 Gaucher disease

OBJECTIVES

- Safety
- Engraftment
- Efficacy (functional endpoints and biomarkers)

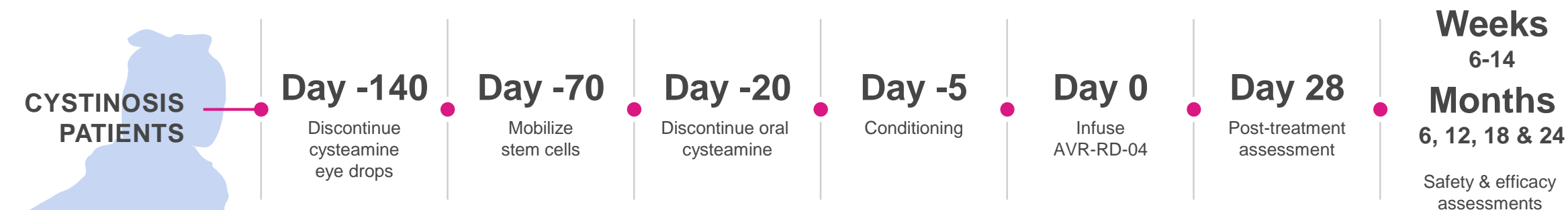
PATIENTS

- 8-16 patients
- 16-35 year old males and females
- Two arms
 - Treatment naïve
 - Stable receiving ERT

ASSESS

- Vector Copy Number (VCN)
- Chimerism
- GCase activity, including in CSF
- Efficacy
 - Hematologic values
 - End-organ volumes and BMD
 - Biomarkers and QoL
- Safety

Investigator-sponsored* Phase 1/2 study in Cystinosis



A Phase 1/2 study to determine the safety and efficacy of transplantation with autologous human CD34+ Hematopoietic Stem Cells (HSC) from Mobilized Peripheral Blood Stem Cells (PBSC) of patients with Cystinosis modified by ex vivo transduction using the pCCL-CTNS lentiviral vector

OBJECTIVES	PATIENTS	ASSESS
<ul style="list-style-type: none"> Safety Efficacy 	<ul style="list-style-type: none"> 6 patients adults and potentially adolescents 14–17 years old Using oral and ophthalmic cysteamine 	<ul style="list-style-type: none"> Cystine levels in granulocytes Vector Copy Number (VCN) Chimerism Renal, respiratory and endocrine function, ophthalmologic findings, muscle strength, growth, bone density, neurologic and psychometric measures Safety

* Sponsored by UCSD



Pompe preclinical program advancing

Integrated 3-part approach

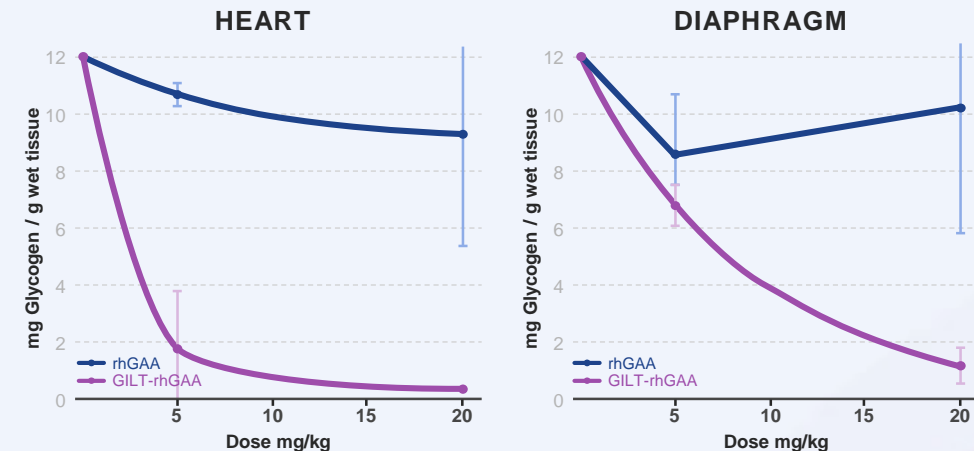
THE CHALLENGE

- Pompe requires **20x more ERT** than Fabry or Gaucher
- Requires GAA activity restored to **muscle and CNS**

AVROBIO's APPROACH

1. Potent transgene promoter
2. GILT uptake tag
3. plato™ for CNS impact

GILT-tagged Recombinant Human (rh)GAA impacts levels of stored glycogen compared to non GILT-tagged Recombinant Human (rh)GAA in a Pompe mouse model



GILT: Glycosylation-Independent Lysosomal Targeting

Sources: Burton B et al, J Pediatr, 2017; Aulsems M et al, Eur J Hum Genet, 1999; Gungor D et al, Orphanet J Rare Dis, 2011; Maga JA et al, J of Bio Chem, 2013



platoTM

—
AVROBIO's foundation
for worldwide commercialization

Beginning-to-end manufacturing platform

+ Optimized
for performance

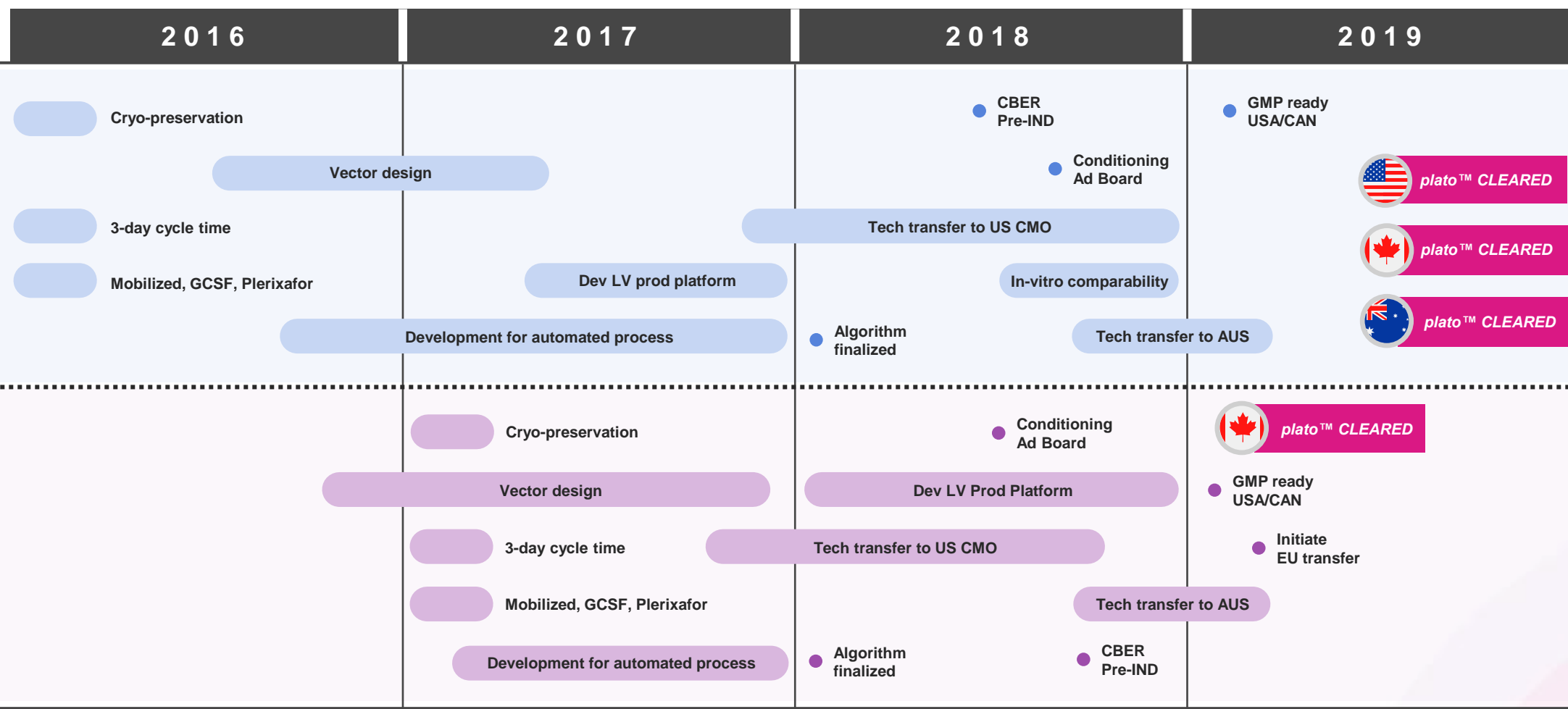
+ Redefines manufacturing
best practices

Multiple plato™ IND and CTA regulatory clearances achieved 1H 2019



FABRY

GAUCHER



Note: plato in Fabry cleared for use in US via IND, in Canada via protocol and CMC CTA amendment, and in AUS via CTN and HREC clearance; plato in Gaucher cleared for use in Canada via CTA and protocol CTA amendment

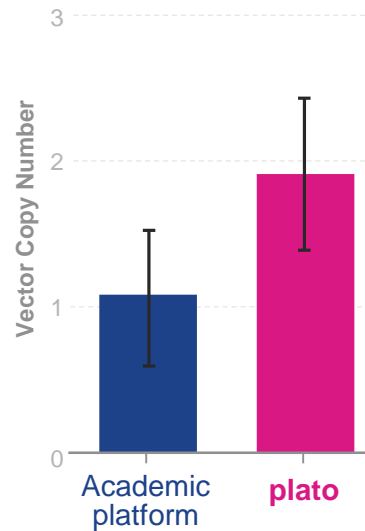
plato™ optimized for performance



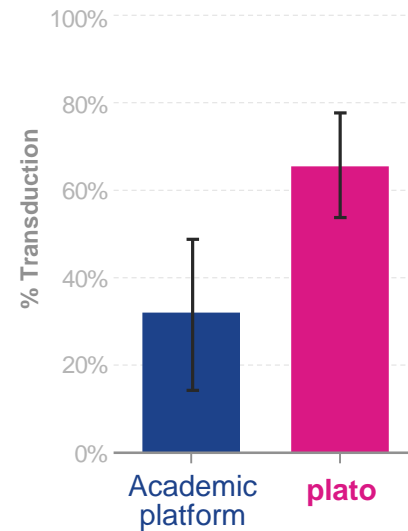
Proprietary Vector Toolbox

- ☒ OPTIMIZED VECTOR
- ☒ PROMOTERS
- ☒ OPTIMIZED TRANSCRIPTION
- ☒ OPTIMIZED TRANSLATION
- ☒ TAGS
- ☒ KOZAK SEQUENCE
- ☒ CODON OPTIMIZATION

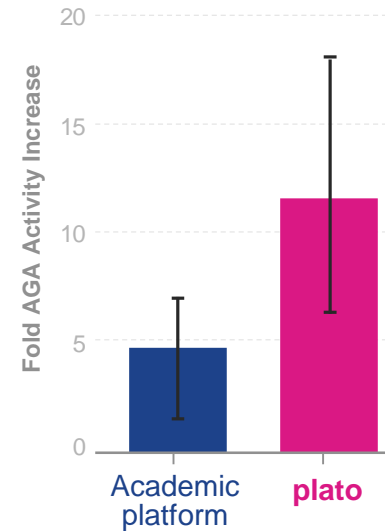
VCN



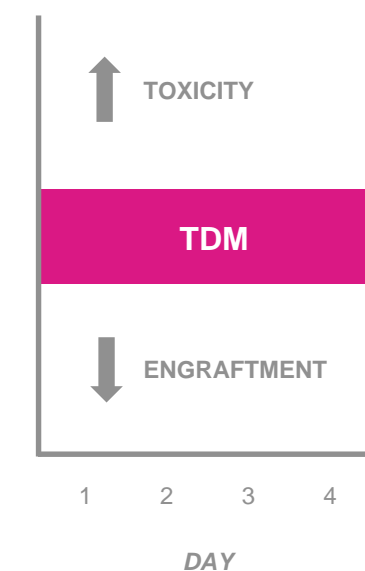
Transduction Efficiency



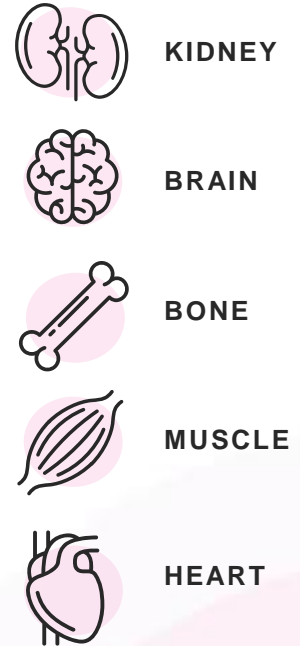
Enzyme Activity



Therapeutic Drug Monitored (TDM) Conditioning

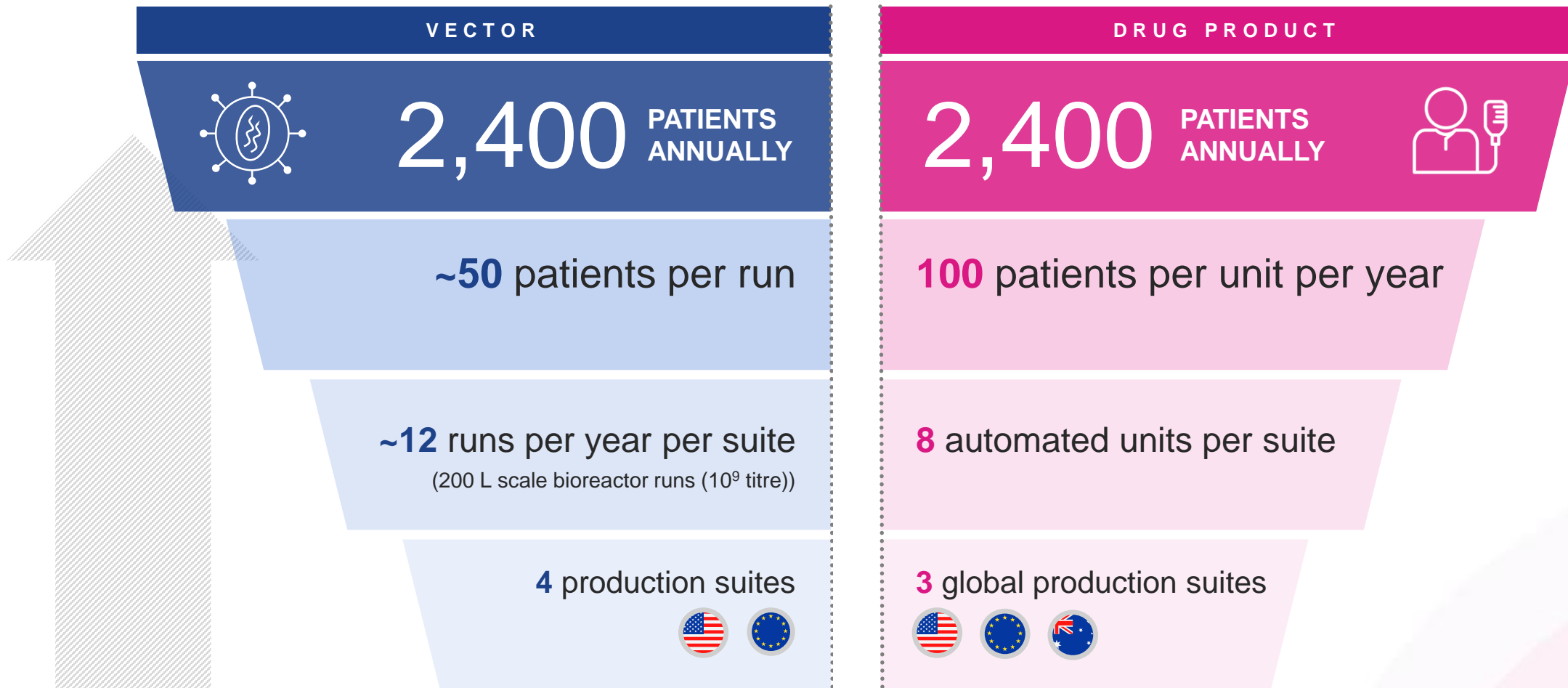


Distribution



Note: Data from appropriate runs from normal donors and patients are included in the analysis; Data cutoff March 12, 2019

plato™ platform designed to be scalable for commercial supply





Multiple 2H 2019 milestones anticipated



FABRY

- Continued recruitment in FAB-201, with plato™ to be incorporated
- FAB-201 clinical sites to expand into USA and Canada



GAUCHER

- Dose first patient in GAU-201, incorporating plato™ from the outset



CYSTINOSIS

- Dose first patient in investigator-sponsored trial



POMPE

- Pre-clinical IND-enabling study to be initiated



Appendix



Precedent for use of kidney biopsy data for FDA approval of drug candidate for Fabry disease

Migalastat approved on % reduction in GL-3 inclusions per KIC as compared to placebo

45 Amenable patients* (16 males / 29 females)

Group	Migalastat (BL –M6)	Placebo (BL –M6)
Males (N=16)	5/7 (71%) -1.10 (-1.94, -0.02)	4/9 (44%) -0.03 (-1.00, 1.69)
Patients with baseline GL-3 ≥ 0.3 (N=17; 9 males, 8 females)	7/9 (78%) -0.91 (-1.94, 0.19)	2/8 (25%) -0.02 (-1.00, 1.69)
Patients with baseline GL-3 < 0.3 (N=28; 7 males, 21 females)	6/16 (38%) -0.02 (-0.10, 0.26)	7/12 (58%) -0.05 (-0.16, 0.14)

7/9 males ≥ 50% reduction
(at 6 months from baseline)

Treatment Group	n	Baseline Median (min, max)	Month 6 Median (min, max)	Change from Baseline Median (min, max)
Average number of GL-3 inclusions per KIC (N=13)				
GalaFold	7	3.6 (0.2, 6.0)	2.6 (0.1, 6.0)	-0.7 (-1.7, 1.2)
Placebo	6	1.8 (0.1, 2.8)	2.0 (0.05, 4.3)	-0.04 (-0.5, 1.5)

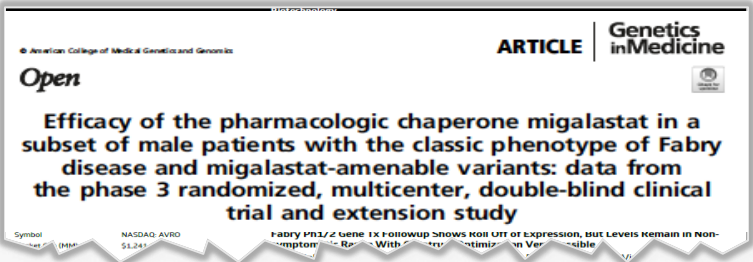
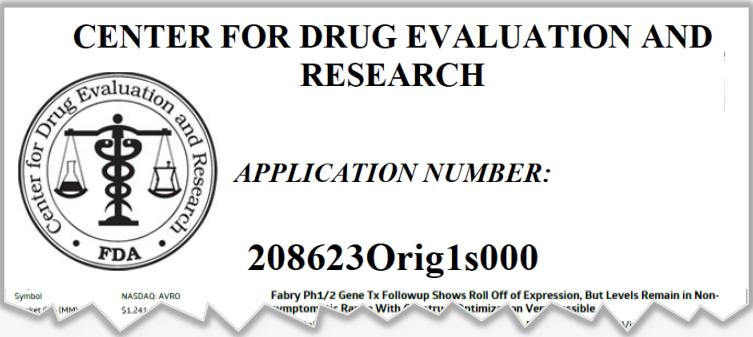
28% average reduction
(at 6 months from baseline)

Classic Fabry patient level data

0-6 months randomized clinical trial and 6-12 months open label extension

	Male Patients with the Classic Phenotype													
	Migalastat (Months 0-24)							Placebo (Months 0-6) → Migalastat (Months 6-24)						
	#1	#2	#3	#4	#5	#6	#7	#8	#9	#10	#11	#12	#13	#14
PTC GL-3 inclusions at BL	0.16	0.03	n/a	5.69	1.22	n/a	2.88	2.41	1.55	0.16	0.03	0.11	0.94	0.88
Change in PTC GL-3 inclusions from BL to M6	-0.08	0.01	n/a	-1.77	-1.10	n/a	-1.25	1.21	-0.21	0.01	0.09	-0.07	1.94	-0.83
Change in PTC GL-3 inclusions from BL/M6 to M12	-0.12	n/a	n/a	-1.92	n/a	n/a	-0.81	-0.94	-1.13	-0.09	-0.05	n/a	-2.28	0.06

46% average reduction
(average of patients with 12 month data)



Source: Germain D et al, Genetics in Medicine, 2019

- Classic Fabry disease (AGA activity <1%)
- **NOTE:** For informational purposes; differences exist between trial designs and subject populations; AVROBIO has not conducted any head-to-head trials comparing migalastat to AVR-RD-01

Hematopoietic reconstitution occurs in two distinct phases

A few thousand long-term engrafting cells stably sustain levels of transgene product

First wave of short-term progenitor cells start to exhaust with progressive takeover by a smaller population of long-term engrafting cells

