AVROBIO

ASGCT 2020

Fabry & Cystinosis Data Update

May 13, 2020

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ASGCT 2020 data update – key takeaways

New data show consistent results across Fabry disease and cystinosis programs

Long-term Fabry patient data

Sustained long-term positive trends

- Patient 1 in the Phase 2 trial continues to show stable leukocyte and plasma AGA enzyme activity, now out 22 months
- Patient 3 in the Phase 2 trial shows increased leukocyte and plasma AGA enzyme activity, decreased plasma lyso-Gb3 level, and stable VCN at new time points
- All three Phase 1 patients off ERT remain off ERT

First Fabry plato™ patient

plato continues to perform

- One-month plasma lyso-Gb3 decrease of 43% vs. baseline
- Three-month leukocyte and plasma enzyme activity levels 3x greater than mean of other three patients at same timepoint in Phase 2 trial
- Rapid neutrophil and platelet recovery with minimal lymphocyte depletion post Bu90 conditioning

Cystinosis Patient 1 data

Positive trends at six months, including kidney function measures

- eGFR and serum creatinine measures trending positively at 6 months
- Pill burden remains significantly lower than at baseline

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Multiple programs in the clinic

10 patients dosed to date

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

IND: Investigational New Drug

Fabry Disease

AVR-RD-01

Goals for gene therapy in **Fabry disease**

UNMET NEEDS:

Kidney function
Unmet needs: proteinuria, polyuria, kidney failure

Cardiac function
Unmet needs: left ventricular hypertrophy, fibrosis, heart failure

Neuropathic pain
Unmet needs: pain and burning sensations in hands and feet, pain crises

CNS complications
Unmet needs: TIA/stroke, depression, impaired executive function, white matter hyperintensities

Everyday burden of illness and life expectancy
Unmet needs: fatigue, inability to sweat, joint pain, abdominal pain, diarrhea, vomiting, cloudy vision, hearing loss, tinnitus, rash, angiokeratomas, biweekly infusions, shortened lifespan

Sources: Wanner C et al, Med Genetics and Metab, 2018; Burlina A, JIEMS, 2016
CNS: Central Nervous System; TIA: Transient Ischemic Attack

Two AVR-RD-01 Fabry clinical trials

9 patients dosed across Phases 1 and 2

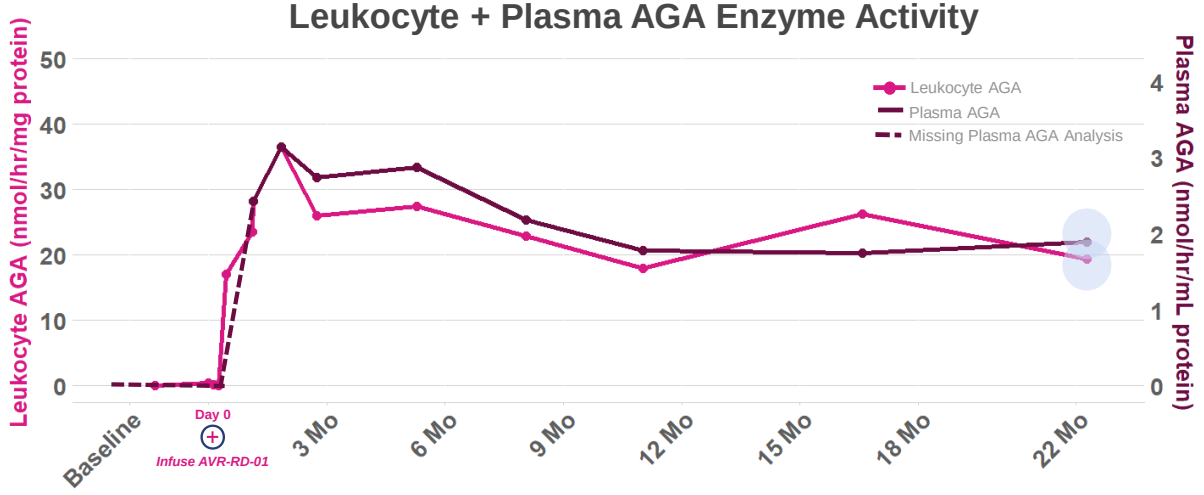
PHASE 1 Investigator-Sponsored Trial*	PHASE 2 AVRO – FAB-201 Trial
Patients **n = 5 (fully enrolled)** **On ERT > 6 months prior to enrollment** **18-50 year-old males**	**Patients** **n = 8-12 (4 patients dosed to-date)** **Treatment-naive** **16-50 year-old males**
Key Objective Safety and preliminary efficacy	**Key Objectives** Safety and efficacy

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

New data point

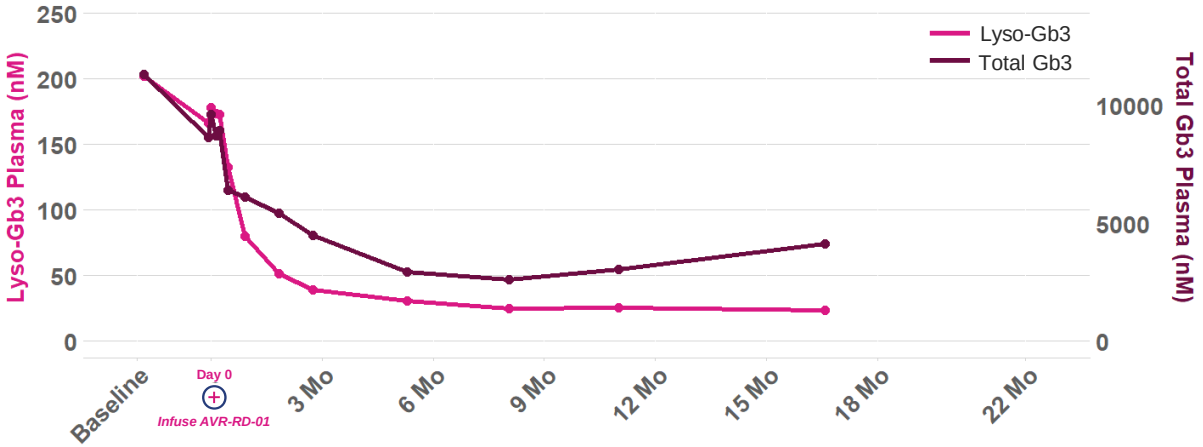
Patient 1: Multiple data trends sustained up to 22 months

Leukocyte + Plasma AGA Enzyme Activity



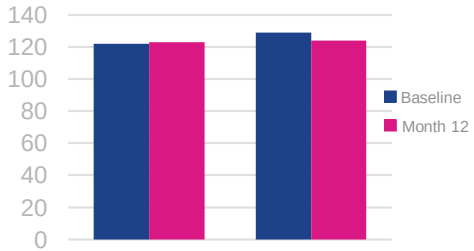
*Lab A: Mayo Clinic Laboratories; Lab B: Rupar Laboratory; Lab A Reference Range: >23.1 nmol/hr/mg; Lab B Reference Range: 24–56 nmol/hr/mg
†Reference Range: 5.1–9.2 nmol/hr/mL; AGA: α-galactosidase A

Plasma Lyso-Gb3 and Total Gb3



*Reference Value: 2.4 nM; †Reference Value: 4961 nM; 6012 nM before August 2018 (until Day 28 for Patient 1)
Lyso-Gb3: Globotriaosylsphingosine; Gb3: Globotriaosylceramide

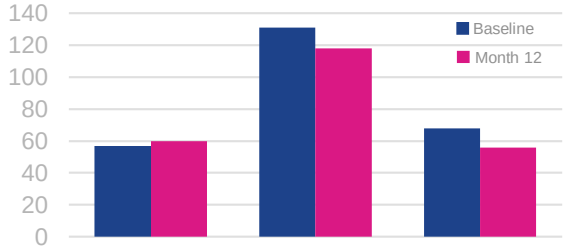
KIDNEY FUNCTION
remains within normal range at 12 mos.



	mGFR/eGFR	
Normal Range	Average 116* mL/min/1.73 m²	
	Male (20–39 years)	

***Source**: https://www.kidney.org/atoz/content/gfr*
mGFR: Measured Glomerular Filtration Rate; eGFR: Estimated Glomerular Filtration Rate

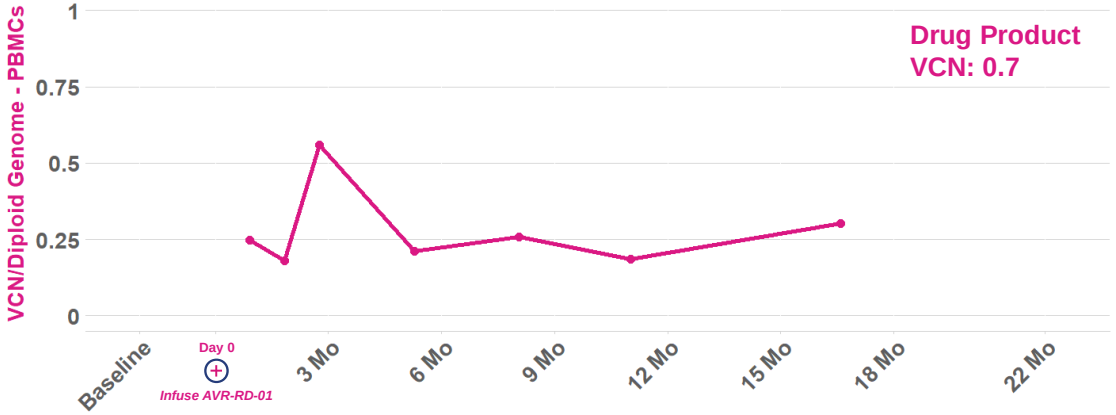
CARDIAC FUNCTION
remains within normal range at 12 mos.



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

***Source:** Alfakih K et al, J Magn Reson Imaging, 2003*
EF: Ejection Fraction; LV: Left Ventricular

Vector Copy Number (VCN)

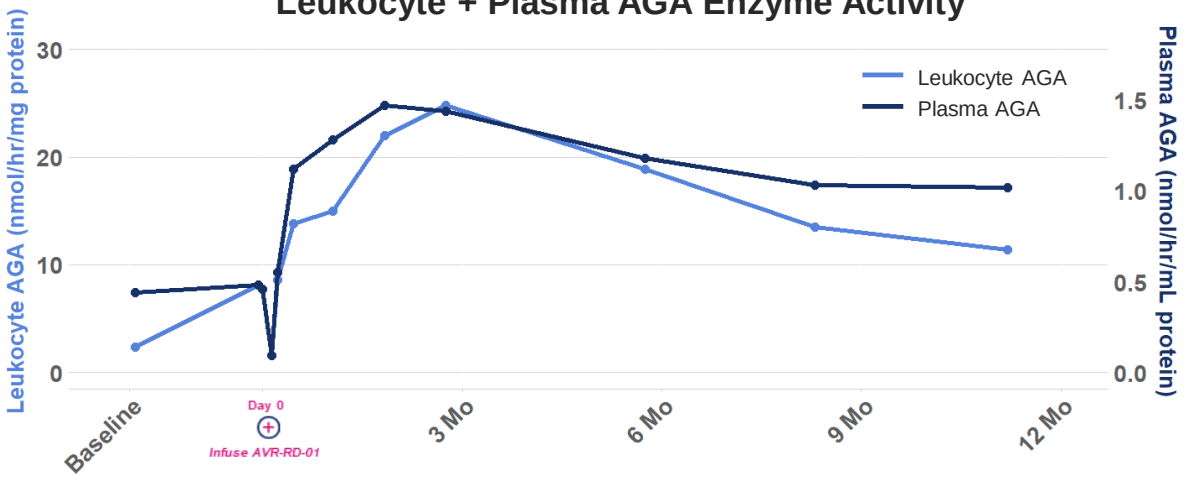


VCN: Vector Copy Number; PBMCs: Peripheral Blood Mononuclear Cells

Note: Patient #1 had a skin biopsy score of 3 (severe accumulation) at baseline, a score of 2 (moderate accumulation) at 6 months and a score of 1 (mild accumulation) at 12 months
Note: Visualization of multiple biomarkers adjusted to utilize the same statistical scaling ratio across biomarkers (as compared to prior presentation)

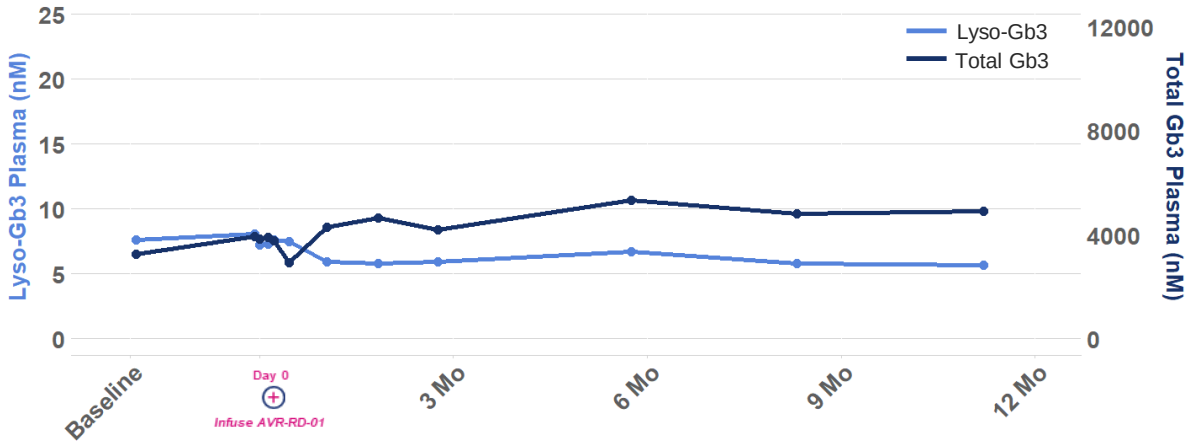
Patient 2: Multiple data trends sustained up to 1 year*

Leukocyte + Plasma AGA Enzyme Activity



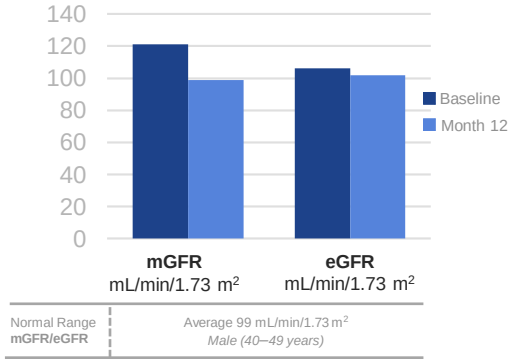
*Data from Rupar Laboratory; Reference Range: 24–56 nmol/hr/mg; †Reference Range: 5.1–9.2 nmol/hr/mL; AGA: α-galactosidase A

Plasma Lyso-Gb3 and Total Gb3



*Reference Value: 2.4 nM; †Reference Value: 4961 nM; **Note:** Patient #2 has normal substrate, consistent with late-onset cardiac variant phenotype
Lyso-Gb3: Globotriaosylsphingosine; Gb3: Globotriaosylceramide

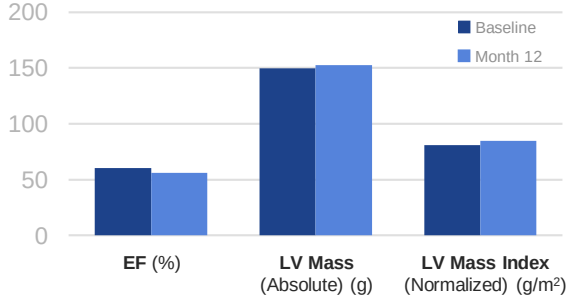
KIDNEY FUNCTION *remains within normal range*



Normal Range mGFR/eGFR	Average 99 mL/min/1.73 m² Male (40–49 years)

Source: https://www.kidney.org/atoz/content/gfr
mGFR: Measured Glomerular Filtration Rate; eGFR: Estimated Glomerular Filtration Rate

CARDIAC FUNCTION *remains within normal range*



Reference Range Mean Values Male 40-49 years	55–65%	58–91 g/m²

Source: Alfakih K et al, J Magn Reson Imaging, 2003
EF: Ejection Fraction; LV: Left Ventricular

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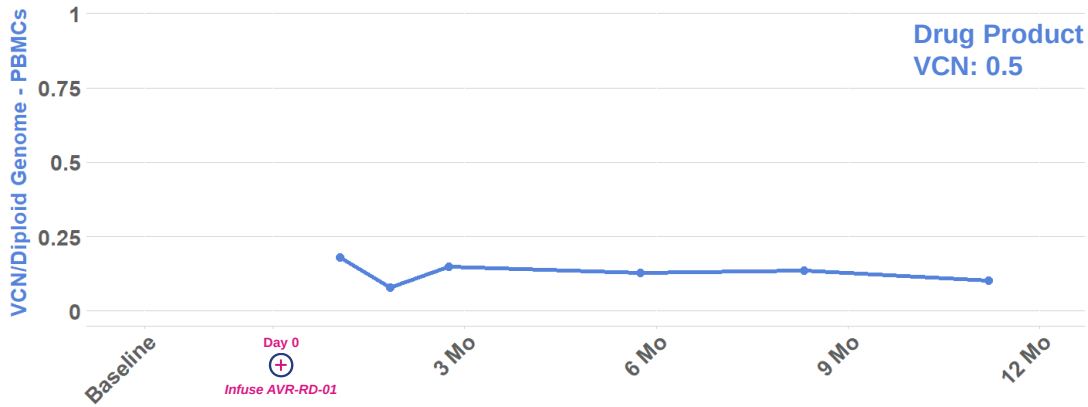

VCN: Vector Copy Number; PBMCs: Peripheral Blood Mononuclear Cells

Note: As patient #2 is a cardiac variant of Fabry disease, this patient had a skin biopsy score of 0 (trace or no accumulation) at baseline and at 6 months
Note: Visualization of multiple biomarkers adjusted to utilize the same statistical scaling ratio across biomarkers (as compared to prior presentation)
** Latest data points for this patient are at the 1-year follow-up which = 48 weeks per protocol*

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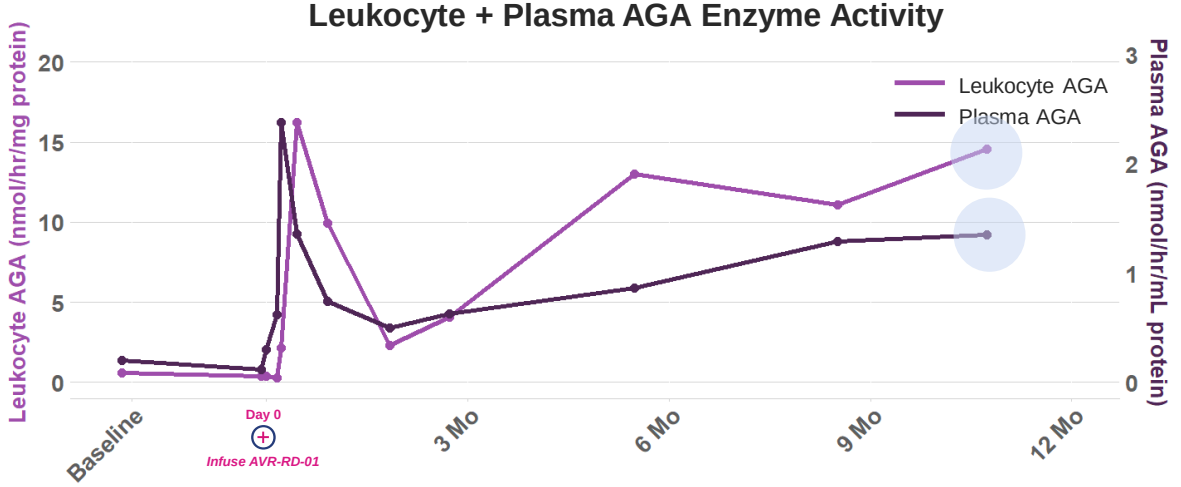
New data point

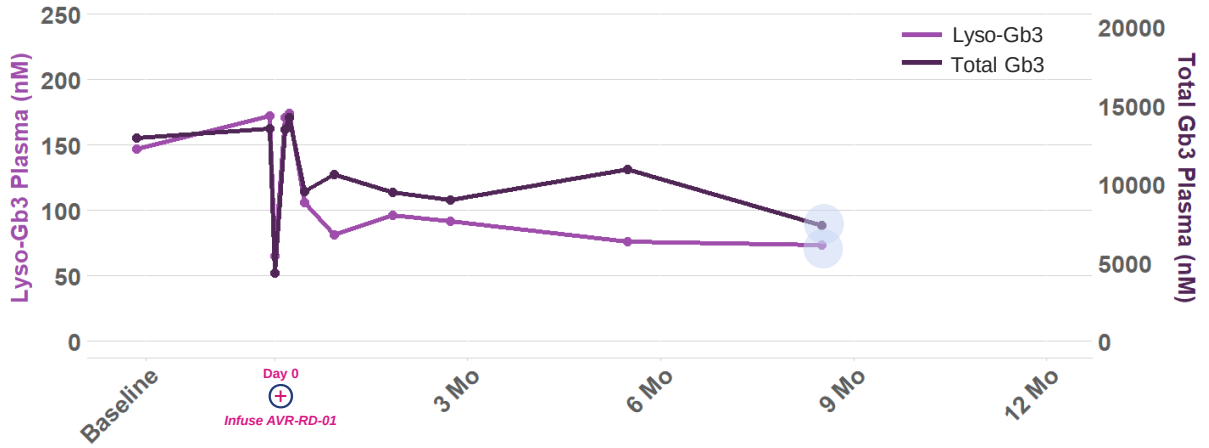
Patient 3: Data up to 1 year* suggest trend towards durable engraftment

Leukocyte + Plasma AGA Enzyme Activity



*Data from Rupar Laboratory; Reference Range: 24–56 nmol/hr/mg; †Reference Range: 5.1–9.2 nmol/hr/mL; AGA: α-galactosidase A

Plasma Lyso-Gb3 and Total Gb3

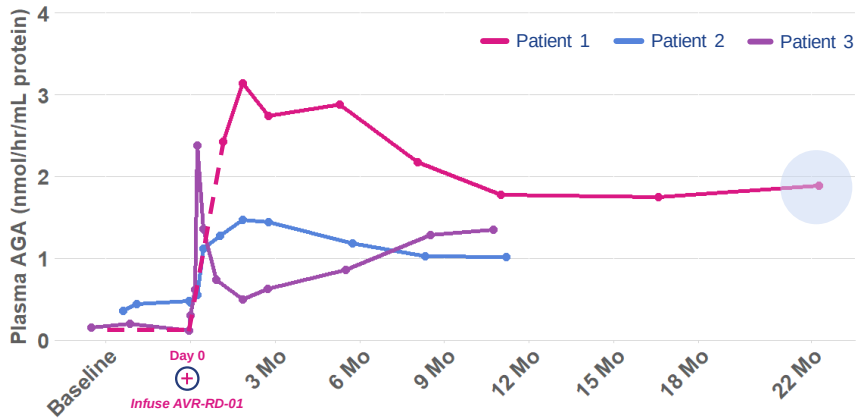


*Reference Value: 2.4 nM; †Reference Value: 4961 nM; Lyso-Gb3: Globotriaosylsphingosine; Gb3: Globotriaosylceramide

Skin Biopsy Score (Patient 3)

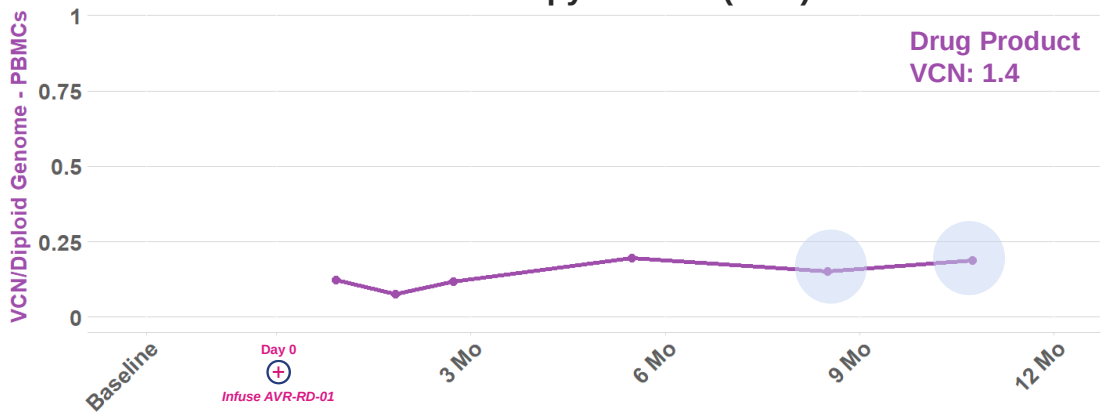
Baseline	2
6 months	2

Plasma AGA Enzyme Activity



Reference Range: 5.1–9.2 nmol/hr/mL; AGA: α-galactosidase A

Vector Copy Number (VCN)



VCN: Vector Copy Number; PBMCs: Peripheral Blood Mononuclear Cells

Note: Visualization of multiple biomarkers adjusted to utilize the same statistical scaling ratio across biomarkers (as compared to prior presentation)

**1-year follow-up = 48 weeks per protocol*

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New data point

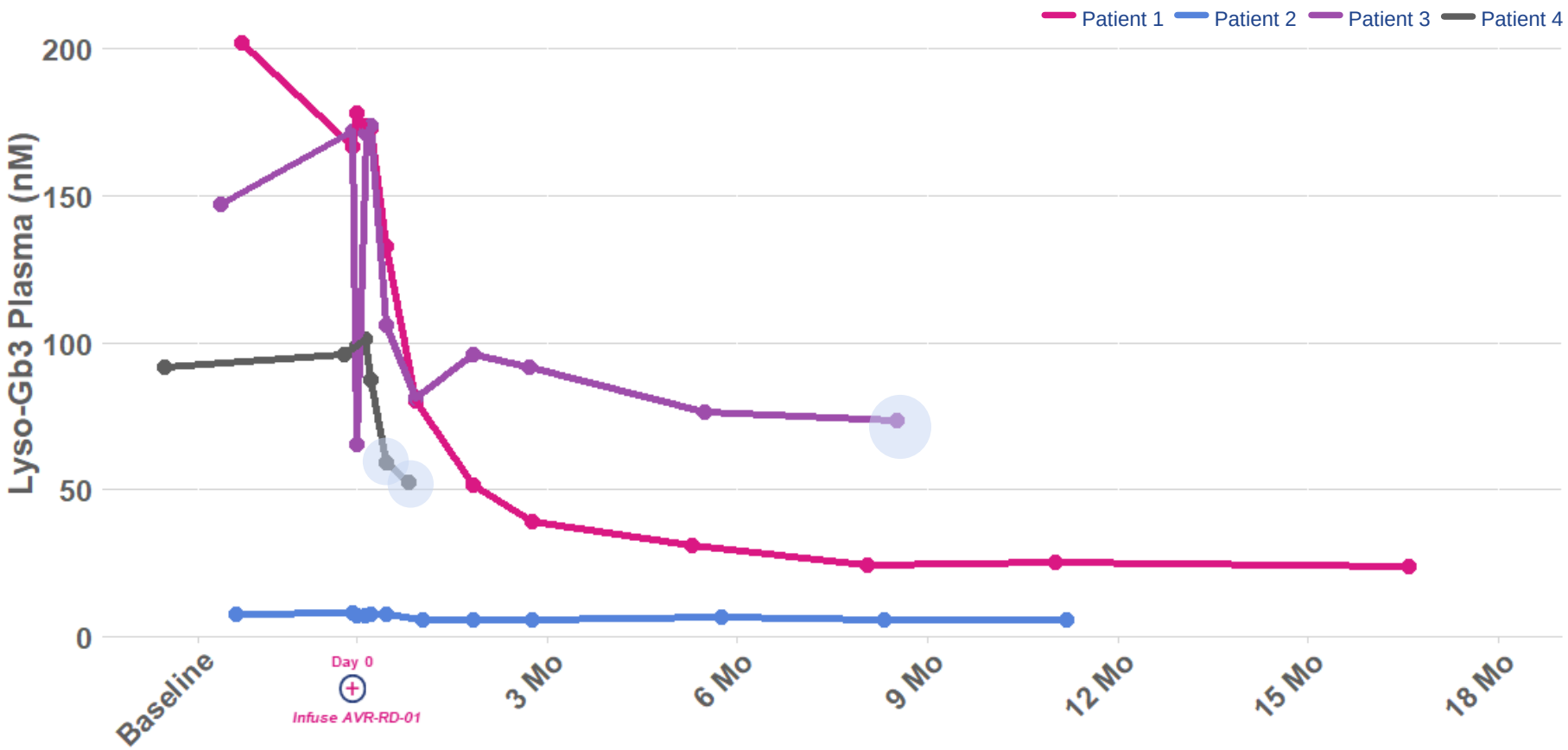
Patients 1-4: Plasma and leukocyte enzyme activity sustained up to 22 months

Patient #4 dosed using plato™

AGA: α-Galactosidase A

New data point

Patients 1-4: Plasma lyso-Gb3 reduction sustained up to 18 months



Reduction from Baseline to Last Observation	
Patient 1	88%
Patient 2	NA
Patient 3	50%
Patient 4	43%

- Lyso-Gb3: Globotriaosylsphingosine
- Note: Patient #2 has normal substrate, consistent with late-onset cardiac variant phenotype

Two AVR-RD-01 Fabry clinical trials

9 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 Objectives

Safety and preliminary efficacy

PHASE 2
AVRO – FAB-201 Trial

Patients

n = 8-12 (4 patients dosed to-date}
Treatment-naive
16 - 50 year-old males

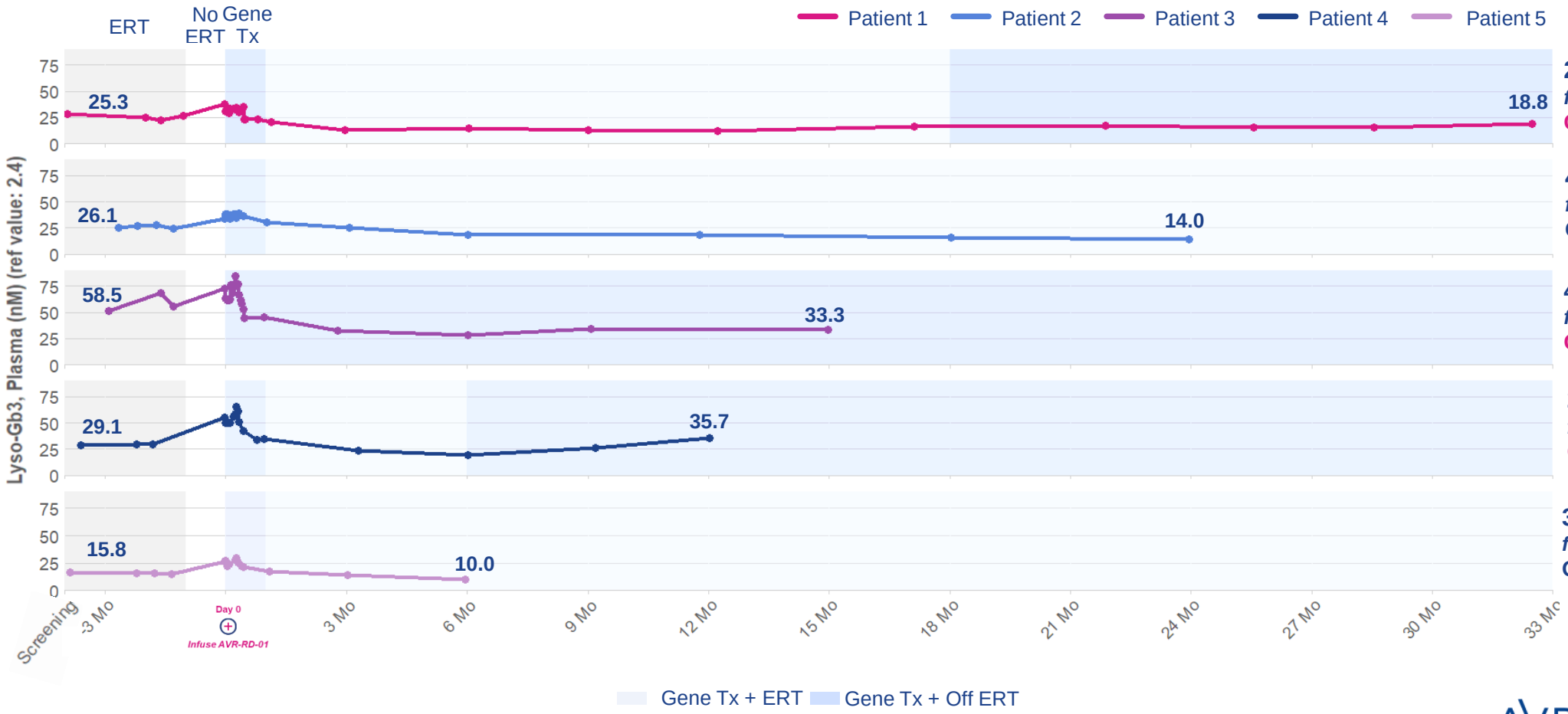
Key Objectives

Safety and efficacy

FAB-201 = AVRO-RD-01-201 Study
** Sponsored by FACTs team (Fabry Disease Clinical Research and Therapeutics) in Canada*
ERT: Enzyme Replacement Therapy

Patients 1-5: Plasma lyso-Gb3 reduction sustained up to 32 months

All patients who have discontinued ERT remain off ERT*



26% reduction *from baseline ERT* **OFF ERT**

47% reduction *from baseline ERT* **ON ERT**

43% reduction *from baseline ERT* **OFF ERT**

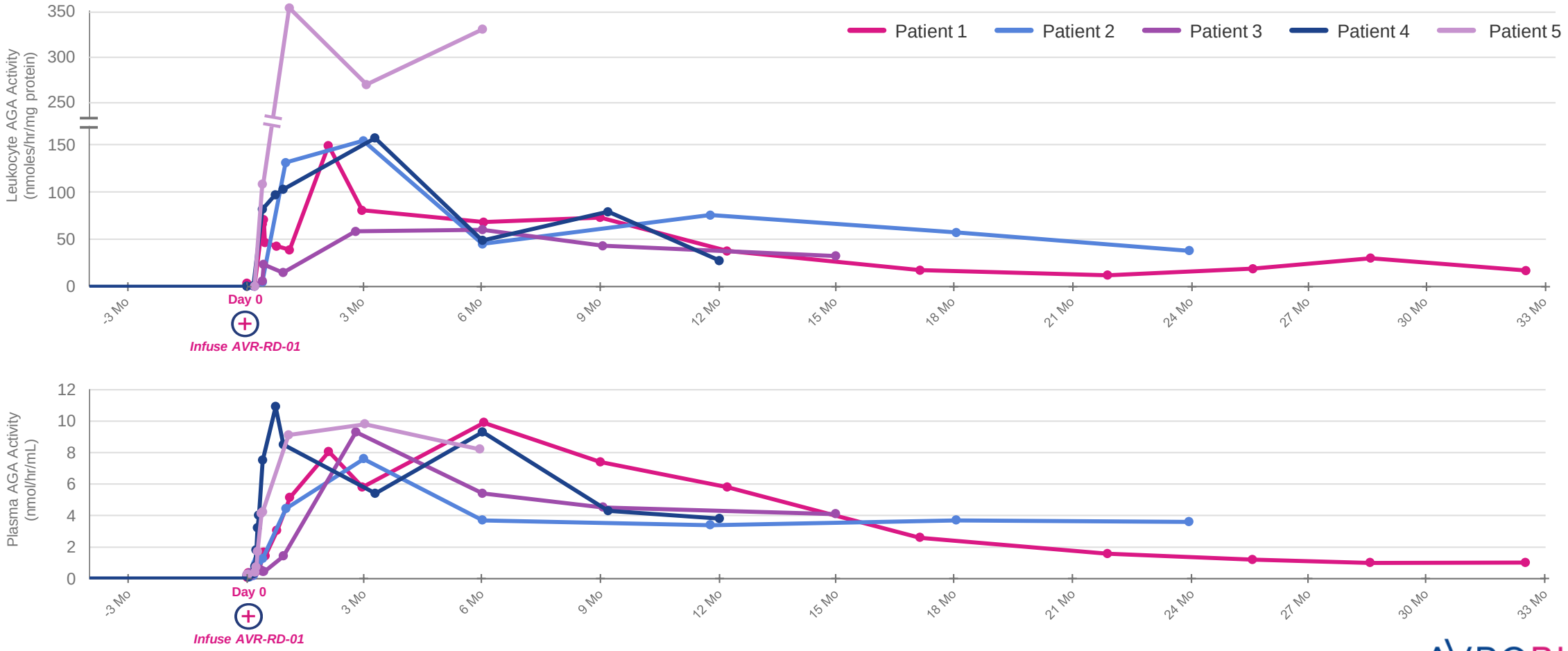
23% increase *from baseline ERT* **OFF ERT**

37% reduction *from baseline ERT* **ON ERT**

** As of April 27, 2020 (update)*
Lyso-Gb3: Globotriaosylsphingosine; ERT: Enzyme Replacement Therapy; Tx: Therapy

Patients 1-5: Leukocyte and plasma enzyme activity sustained up to 32 months

Consistent trends across all patients, 4 patients > 1 year

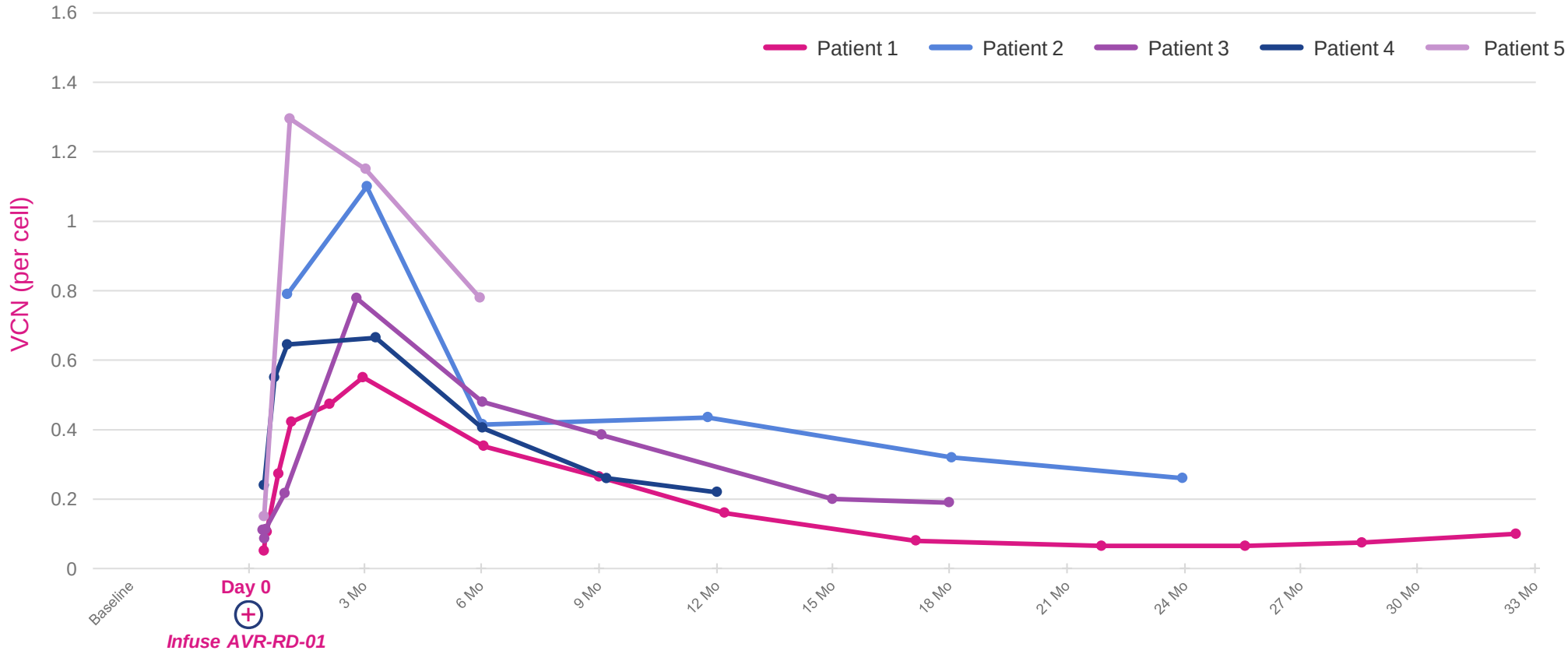


AGA: α-Galactosidase A

VCN stable at 32 months with consistent trend across all other patients

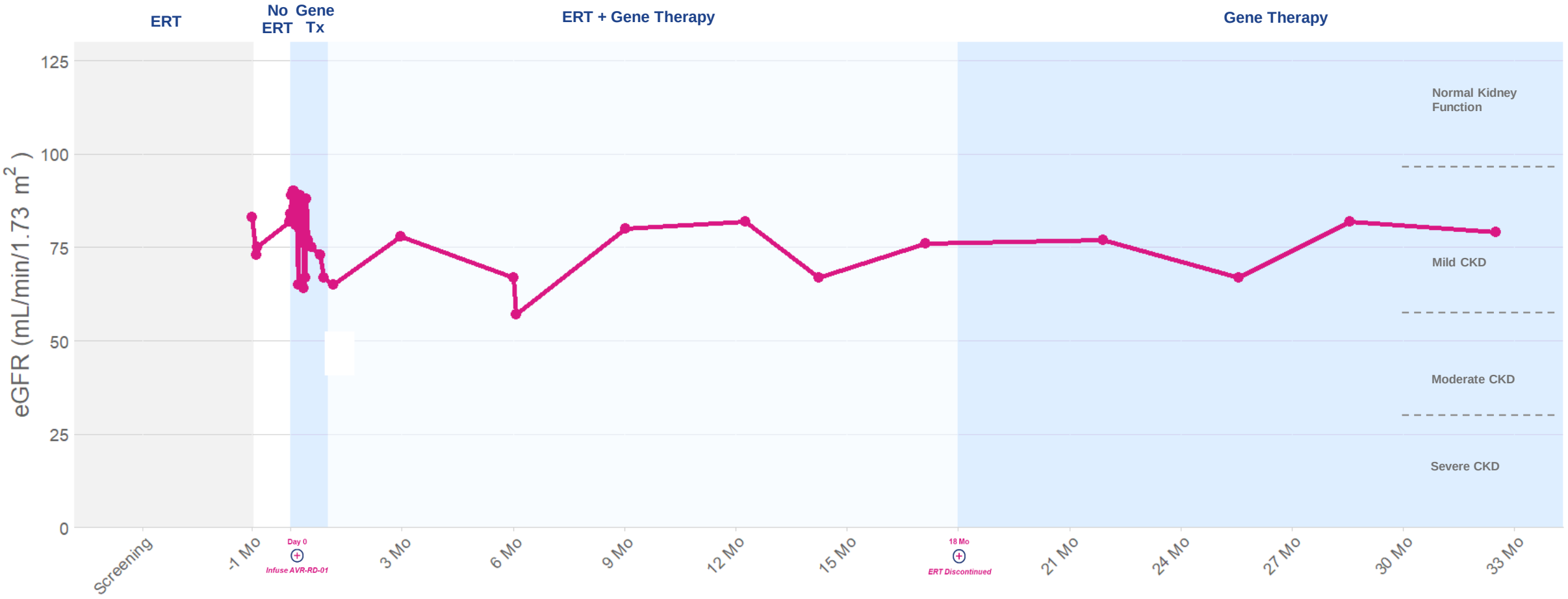
4 patients with 1+ years data

Drug Product VCN	
Patient 1	0.7
Patient 2	1.4
Patient 3	0.8
Patient 4	1.4
Patient 5	1.2



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

Patient 1: Kidney function stable at 32 months



eGFR: Estimated Glomerular Filtration Rate; ERT: Enzyme Replacement Therapy; TX: Therapy; CKD: Chronic Kidney Disease

Phase 1 Fabry (5 patients) and FAB-201 (4 patients)

No unexpected safety events or trends identified

No SAEs related to AVR-RD-01 drug product

AEs and SAEs reported

Phase 1 AEs (n = 128):

- Generally consistent with myeloablative conditioning, underlying disease or pre-existing conditions

Phase 1SAEs (n = 2):

- Febrile neutropenia (grade 3)
- Thrombophlebitis (grade 2)

FAB 201 AEs (n = 98):

- Generally consistent with myeloablative conditioning, underlying disease or pre-existing conditions
  - Grade 1 or 2 (n = 72)
  - Grade 3 or 4 (n = 30)

FAB 201 SAEs: (n = 4)

Pre-treatment and prior to conditioning

- Seizure (grade 2)

Post-treatment

- Dehydration, nausea, vomiting (grade 3)
- Febrile neutropenia (2 patients, grade 3 & 4)

Anti-AGA antibodies

- Pre-existing low titers detected in 4 patients

Note: Safety data cut November 26, 2019
AE: Adverse Event; SAE: Serious Adverse Event
NOTE: AVR-RD-01 is an investigational gene therapy

Cystinosis

AVR-RD-04

Goals for gene therapy in **cystinosis**

UNMET NEEDS:

Kidney function
Unmet needs: renal Fanconi syndrome, proteinuria, chronic kidney disease, kidney failure

Vision
Unmet needs: corneal cystine accumulation, photophobia, involuntary eyelid closure

Endocrine disorders
Unmet needs: softening/weakening of bones, bone pain, rickets, long bone deformations, hypophosphatemia, delayed growth, hypothyroidism, pancreatic insulin insufficiency, diabetes, infertility

CNS complications
Unmet needs: myopathy, hypotonia, tremors, difficulty swallowing, neurodevelopmental issues (speech and walking delay and cognitive impairment)

Everyday burden of illness and life expectancy
Unmet needs: medications multiple times per day that cause GI discomfort and sulfur body and breath smell, shortened lifespan

Sources: Ariceta G et al, Nephrol Dial Transplant, 2015; Elmonem M et al, Orphanet Journal of Rare Diseases, 2016; Gahl et al, NEJM, 2002; Bois et al, J Med Genet, 1976
CNS: Central Nervous System; GI: Gastrointestinal

Investigator-sponsored* study of AVR-RD-04 in cystinosis patients

First patient dosed

PHASE 1/2
Investigator-Sponsored Trial*

Patients

Up to 6 patients
Adults and adolescents
Cohorts 1-2 ≥18 years; Cohort 3 ≥14 years
Male and Female
On oral and ophthalmic cysteamine

Key Objectives

Safety and efficacy

** Sponsored by University of California, San Diego*
Note: AVR-RD-04 aka CTNS-RD-04

Cystinosis AVR-RD-04 Phase 1/2 Patient Characteristics

	PATIENT 1
Age of symptom onset / diagnosis	0 year / 8 months
Age dosed with AVR-RD-04	20 years
Gender	Male
Mutation	Allele 1: LDM_1 Allele 2: Nt1035 (insC)
Primary disease signs and SoC treatment related symptoms, including	• Fanconi syndrome • Polyuria • Corneal abnormalities • Mild photophobia • Vomiting
Granulocyte Cystine levels at baseline (nmol half cystine per mg protein)*	7.8
Comments	NO kidney transplant • Cysteamine 1125 mg p.o. every 12 h/day since 2009; discontinued prior to AVR-RD-04 infusion • Cysteamine eyedrops 4-5x/day • Concomitant medications not listed

Note: AVR-RD-01 aka CTNS-RD-04

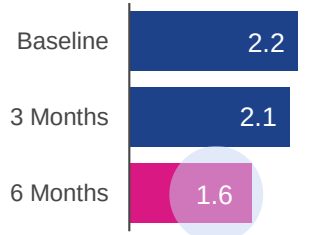
New data point

Patient 1: Initial data indicate positive trends across multiple measures

CLINICAL LAB MEASURES

Kidney Function

eGFR (mL/min/1.73m²)
normal range: >90



Serum Creatinine (mg/dL)
normal range: 0.7-1.2

Urine Volume
24 Hour Urine Volume in L

BIOMARKER ENDPOINTS

Levels of Cystine in Skin*
µm³

- Experimental *in vivo* confocal microscopy
- Two skin areas, behind the ear and 'optional', averaged
- Analysis and quantification (3D Image-Pro software)

VCN
(vcn/dg)
(Drug Product = 2.1)

1 Month	**2.9**
2 Months	**3.0**
3 Months	**2.0**

Average Granulocyte Cystine Level
(µmol half cystine/g protein)

Baseline	**7.8**
1 Month	**1.3**
2 Months	**1.5**
3 Months	**1.5**

Asymptomatic Heterozygous Carrier Granulocyte Cystine Range: 0.2 – 1 .9 µmol half cystine/g protein
Source: Gertsman I et al., Clinical Chemistry, 2016
VCN: Vector Copy Number; CTNS: Cystinosin, Lysosomal Cystine Transporter; mRNA: Messenger Ribonucleic Acid; eGFR: Estimated Glomerular Filtration Rate; SCr: Serum Creatinine
**Data obtained using a novel experimental methodology utilizing in vivo confocal microscopy, to image crystals in the skin behind the ear*

New data point

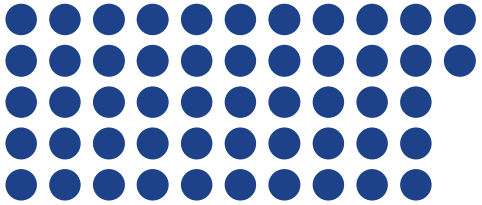
Patient 1: Reduced treatment burden at 6 months

Number of Medications and Supplements

(max per day)

Before Gene Therapy

ON Cysteamine



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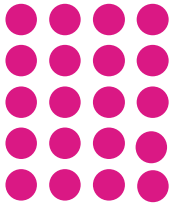
After Gene Therapy

(at 6 months post-gene therapy)

OFF Cysteamine



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NOTE: Investigational gene therapy

Phase 1/2 Cystinosis
1 patient dosed

No unexpected safety events or trends identified

No AEs or SAEs related to AVR-RD-04 drug product

No SAEs reported

AEs reported

- Consistent with myeloablative conditioning and underlying disease
- N = 22 (moderate = 9, mild = 13)

Pre-treatment and prior to conditioning (n = 6, not all events listed)

- Diarrhea, hypokalemia, dizziness
- Dehydration, vomiting

Post-treatment (n = 16, not all events listed)

- Alopecia, intermittent diarrhea, vomiting
- Mucositis, intermittent febrile neutropenia, intermittent epistaxis
- Intermittent blurry vision, intermittent hypokalemia, mucoceles
- Thrombocytopenia

Note: Safety database cut as of January 27, 2020
AE: Adverse Event; SAE: Serious Adverse Event

plato™

AVROBIO's foundation designed to scale gene therapy worldwide

State-of-the-art technologies including automated manufacturing platform

- **Optimized** for performance
- **Redefines** manufacturing best practices

POWERED BY AVROBIO plato™

plato™: Three upgrades designed to optimize potency, safety and durability

UPGRADES	Increase enzyme activity	Increase transduction efficiency	Increase VCN	Increase marrow space / engraftment	Increase consistency and safety
1 | Vector	+	+	+		
2 | Conditioning			+	+	+ *
3 | Automation	+				+

Upgrades designed to increase Vector Copy Number (VCN), enzyme activity, chimerism and durability

* TDM (therapeutic drug monitoring)

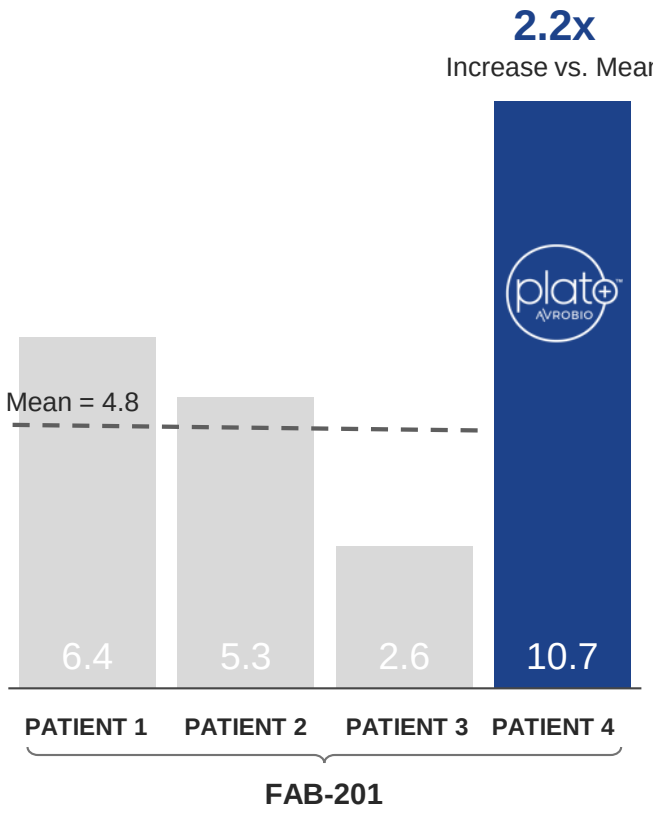
plato™ UPGRADE 1

VECTOR UPGRADE:

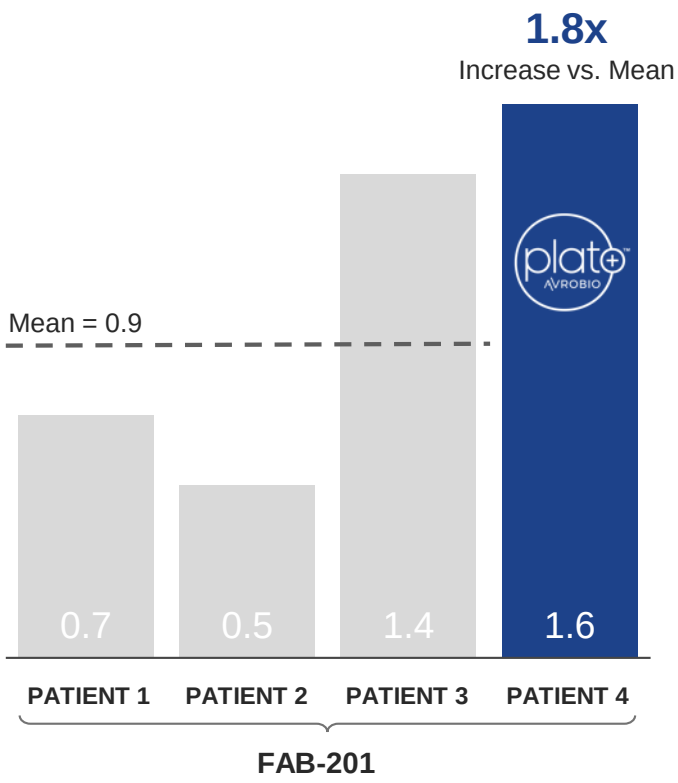
Metrics compared to academic process

FAB-201 patient #4 drug product data with plato™

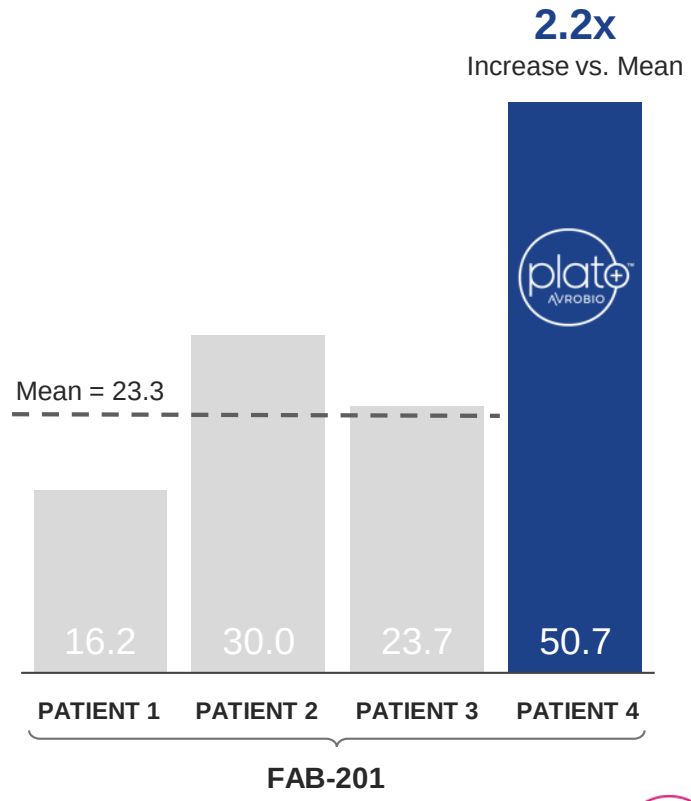
Enzyme Activity (nmol/hr/mL)



VCN (per diploid genome)



Transduction Efficiency (%)



VCN: Vector Copy Number; FAB-201: AVR-RD-01 Study
NOTE: Data is from drug product

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plato™ UPGRADE 1

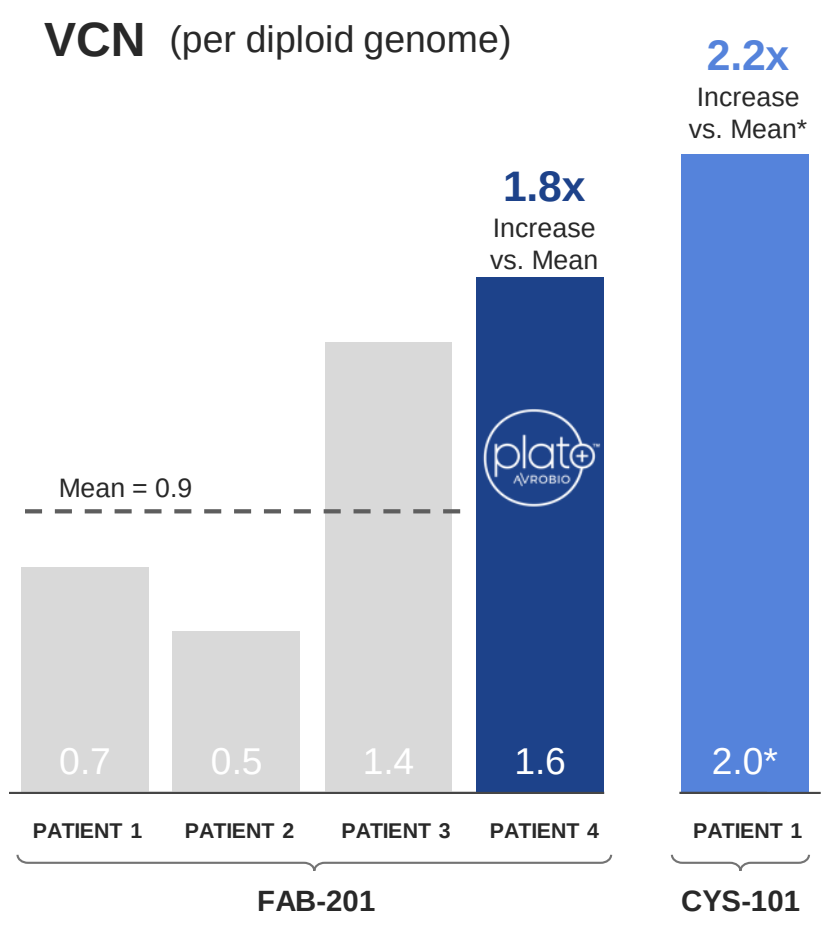
VECTOR UPGRADE:

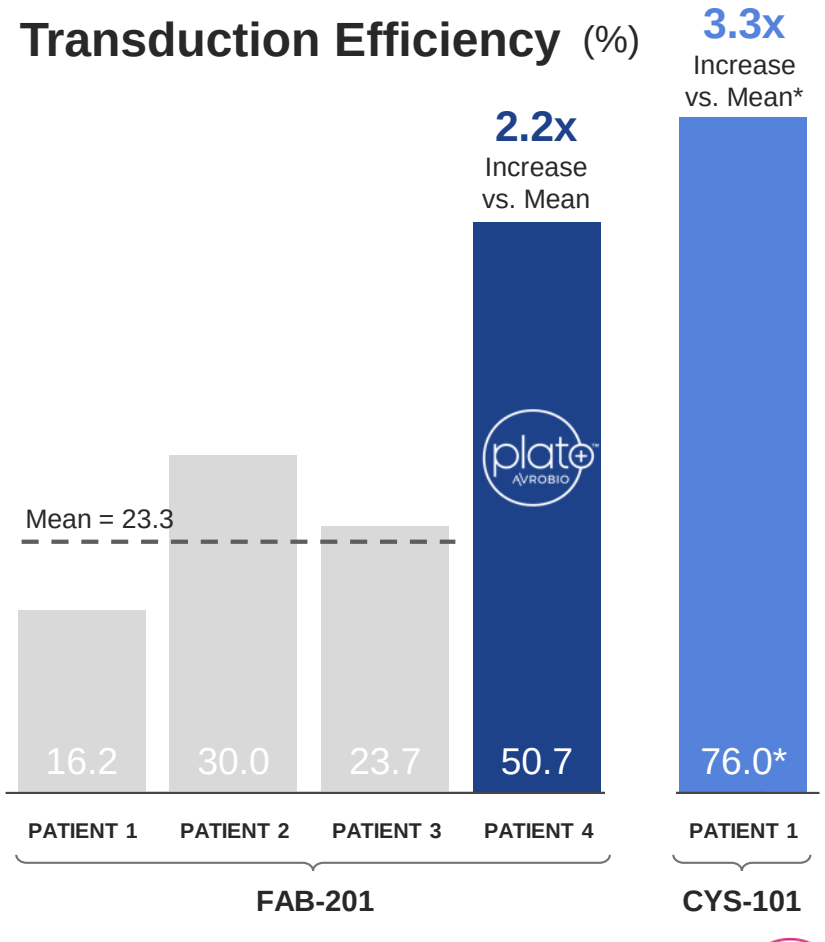
Metrics compared to academic process

FAB-201 and AVR-RD-04 drug product data

FAB-201 with plato™
- 4-plasmid vector (LV2)
- Bu TDM conditioning
- Automated manufacturing

AVR-RD-04 with "plato-like"
- 4-plasmid vector
- Bu TDM conditioning
- Manual manufacturing





BU TDM: Busulfan Therapeutic Drug Monitoring; VCN: Vector Copy Number; FAB-201: AVR-RD-01 Study; CYS-101: AVR-RD-04 Study; LV: Lentiviral Vector
- *Manufactured at UCLA using UCLA's assays and methodologies*

NOTE: Data is from drug product

POWERED BY AVROBIO plato

plato™ UPGRADE 2

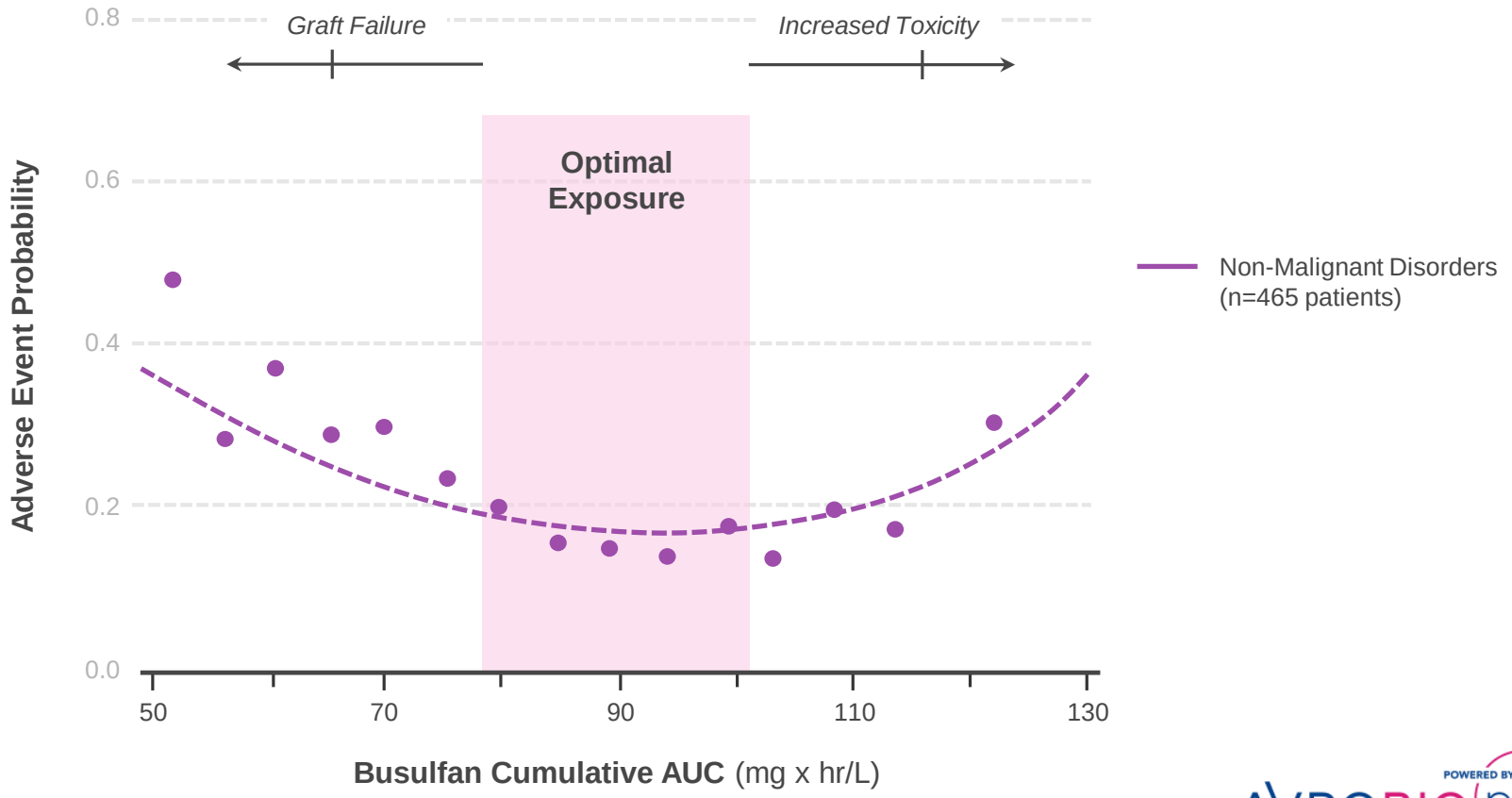
PRECISION CONDITIONING UPGRADE:

Targeted busulfan intended to balance optimal engraftment with enhanced safety

Meta-analysis of 465 patients identified optimal exposure

Optimized precision dosing designed to enhance tolerability

Lowest rate of adverse events in the Bu90 range



Bu: Busulfan; AUC: Area Under the Curve
Sources: Bartelink IH et al, Lancet Haematol, 2016

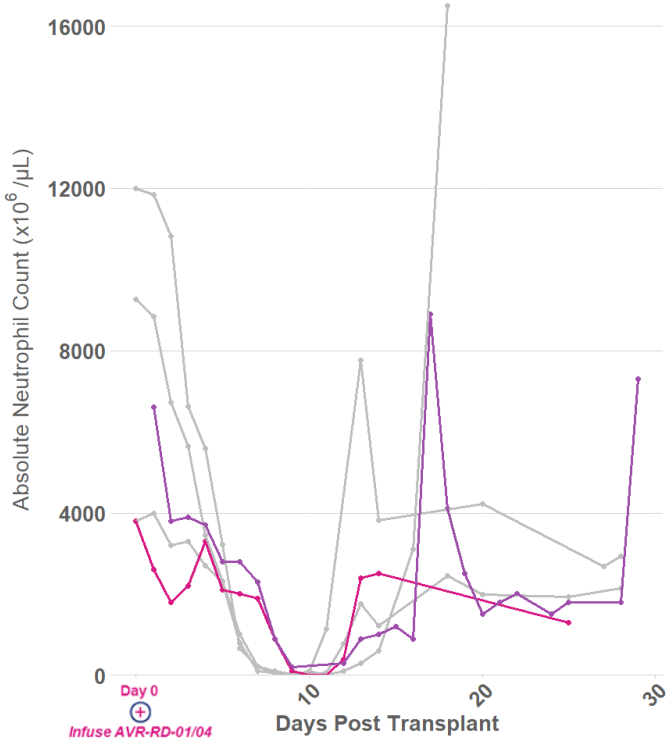
plato™ UPGRADE 2

PRECISION CONDITIONING UPGRADE:

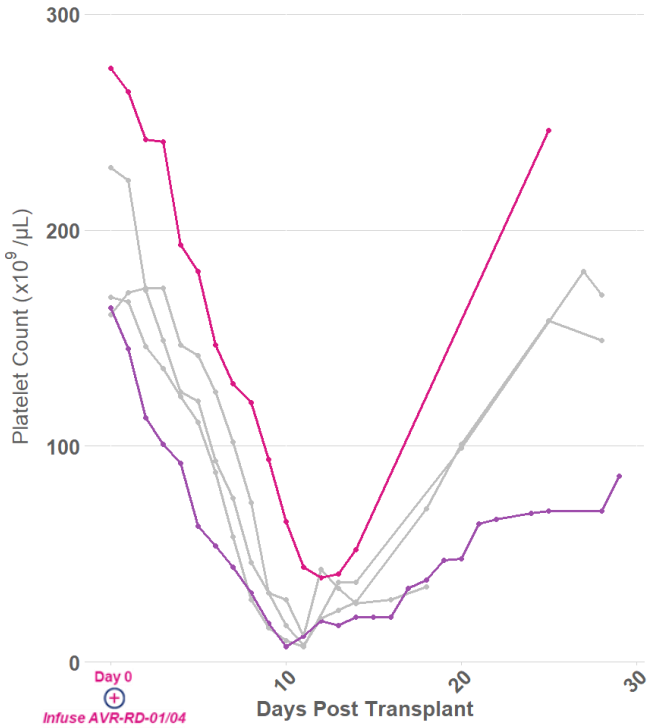
New data point

Rapid neutrophil and platelet recovery with minimal lymphocyte depletion using Busulfan TDM

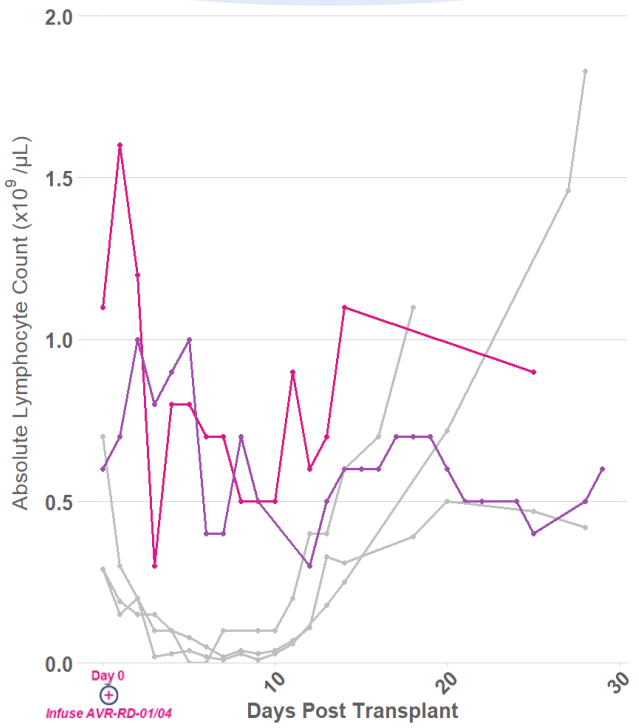
Absolute Neutrophil Count (ANC)



Platelet Count



Absolute Lymphocyte Count



Cystinosis Patient 1: Busulfan | Fabry Patients 1 – 3: Mel | Fabry Patient 4: Bu90-TDM

Fabry: Patients #1-3 Melphalan 100mg/m2; Patient #4 Busulfan 'AUC 90'; Cystinosis: Patient #1 Busulfan 'AUC 90'
Threshold levels for prophylactic supportive care in HSC Tx; ANC <0.5 x 10^9 per liter (AABB); Platelets <10 X 10^9 cells/L (AABB)
NOTE: Neutrophil counts - G-CSF administration post gene therapy: Pt 1: 7 Doses, Day 7 – 14, Pt 2: 11 Doses, Day 7 – 17, Pt 3: 6 Doses, Day 7 – 12, Pt 4: 5 Doses, Day 8 – 12
NOTE: Platelet counts - Platelet Transfusion: Pt 1: Day 10; Pt 2, 3: Day 11, Pt 4: no transfusion
TDM = Therapeutic Drug Monitoring; G-CSF = Granulocyte-colony stimulating factor

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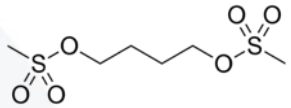
plato™ UPGRADE 2

PRECISION CONDITIONING UPGRADE:

Designed to access “hard-to-reach” compartments

BRAIN

Busulfan crosses blood-brain barrier and eliminates resident microglia cells making space for gene-modified cells



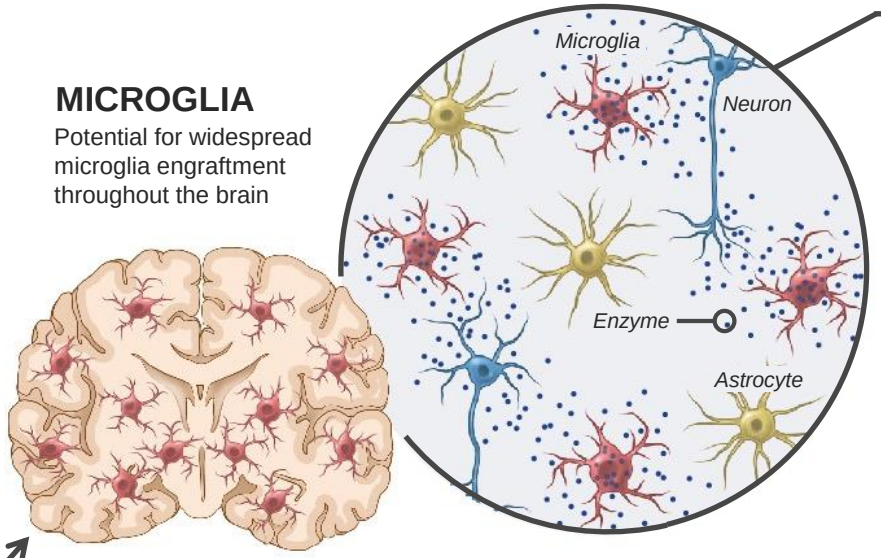
IN THE BONE MARROW

Busulfan eliminates hematopoietic (CD34+) stem and progenitor cells making space for gene-modified cells

TRANSDUCED CD34+ CELLS

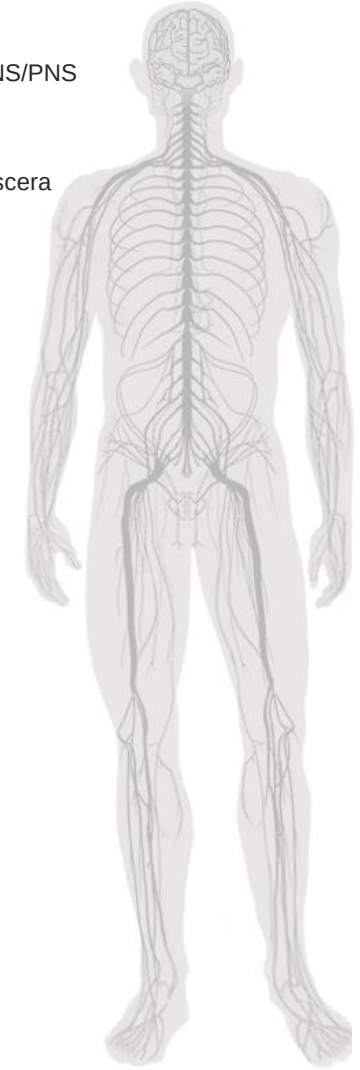
MICROGLIA

Potential for widespread microglia engraftment throughout the brain

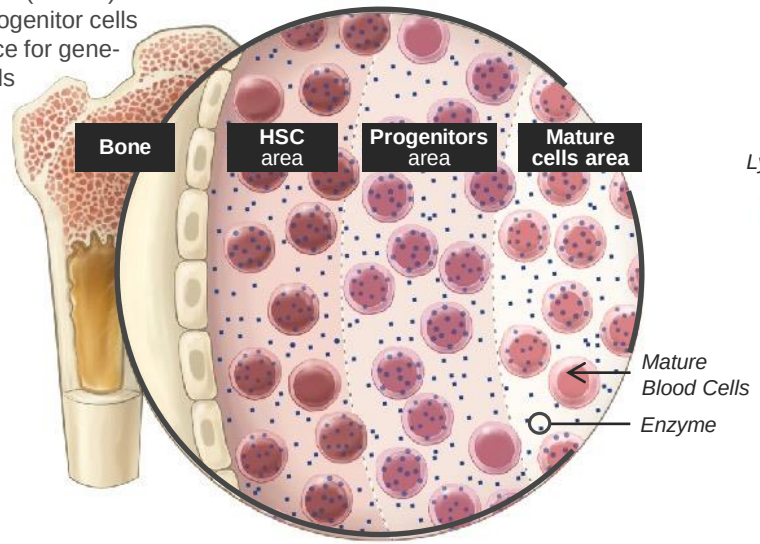


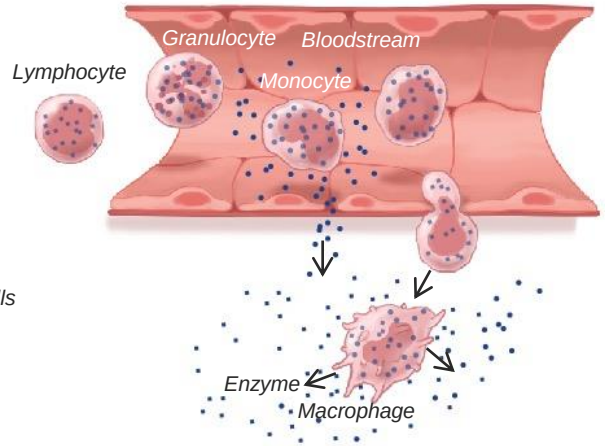
CNS/PNS

Viscera



BONE MARROW → PERIPHERAL TISSUE





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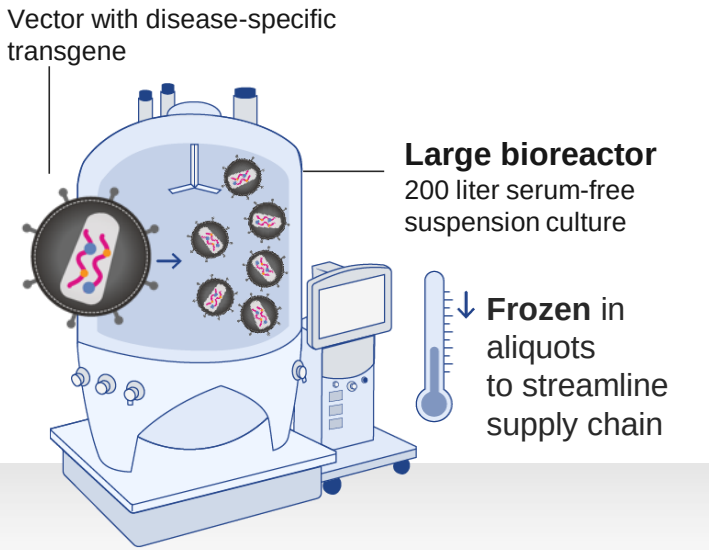
plato™ UPGRADE 3

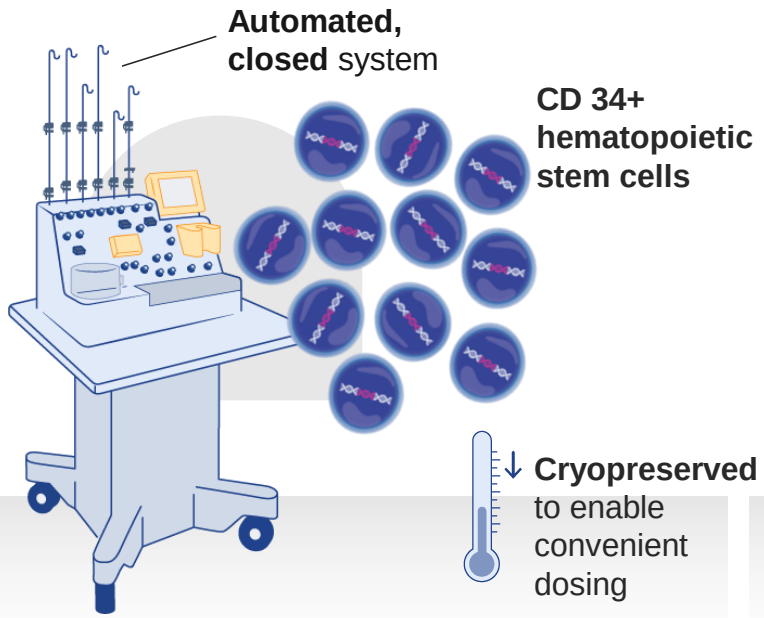
AUTOMATION UPGRADE:

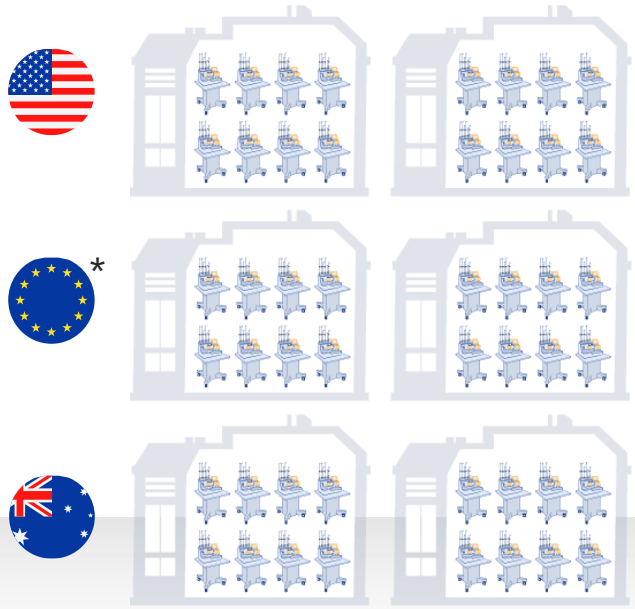
Designed to deliver large-scale manufacturing

Differentiated, cost-effective approach

1 Vector production	2 Drug product production	3 Scalable, global production suites
HIGH VOLUME / TITRE	INCREASE CONSISTENCY	COST-EFFECTIVE SCALE-OUT







Illustrative

* *European manufacturing capabilities planned for 2H 2020; manufacturing capabilities currently in place in U.S. & Australia*

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plato™ UPGRADE 3

AUTOMATION UPGRADE:

Poised to manufacture at scale

Designed to optimize potency and safety, and overcome historic CMC bottlenecks

VECTOR	DRUG PRODUCT
2,400 PATIENTS ANNUALLY	2,400 PATIENTS ANNUALLY
~50 patients per run	**100** patients per unit per year
~12 runs per year per suite (200 L scale bioreactor runs (10^9 titre))	**8** automated units per suite
4 production suites	**3** global production suites

Illustrative

plato™ UPGRADE 1, 2, 3

3 UPGRADES IN PLACE:

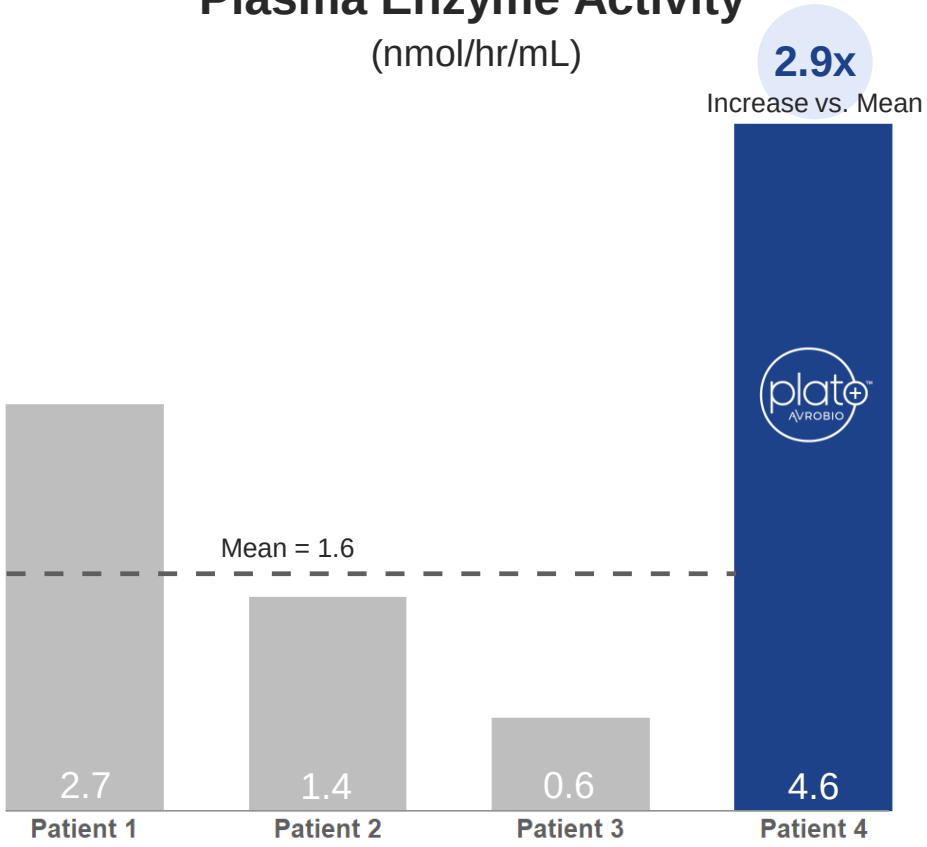
New data point

plato™ metric compared to academic process

FAB-201 THREE MONTH data for patient #4 with plato™ vs. patients #1-3

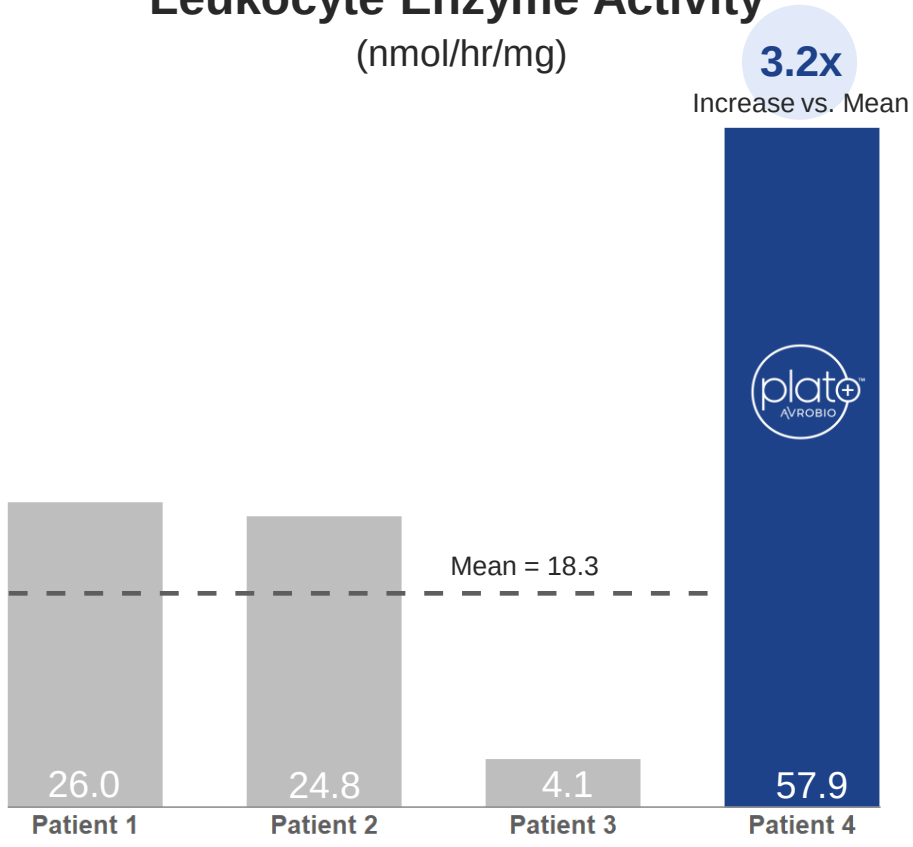
Plasma Enzyme Activity

(nmol/hr/mL)



Leukocyte Enzyme Activity

(nmol/hr/mg)



FAB-201: AVR-RD-01 Study

ASGCT 2020 data update – key takeaways

New data show consistent results across Fabry disease and cystinosis programs

Long-term Fabry patient data

Sustained long-term positive trends

- Patient 1 in the Phase 2 trial continues to show stable leukocyte and plasma AGA enzyme activity, now out 22 months
- Patient 3 in the Phase 2 trial shows increased leukocyte and plasma AGA enzyme activity, decreased plasma lyso-Gb3 level, and stable VCN at new time points
- All three Phase 1 patients off ERT remain off ERT

First Fabry plato™ patient

plato continues to perform

- One-month plasma lyso-Gb3 decrease of 43% vs. baseline
- Three-month leukocyte and plasma enzyme activity levels 3x greater than mean of other three patients at same timepoint in Phase 2 trial
- Rapid neutrophil and platelet recovery with minimal lymphocyte depletion post Bu90 conditioning

Cystinosis Patient 1 data

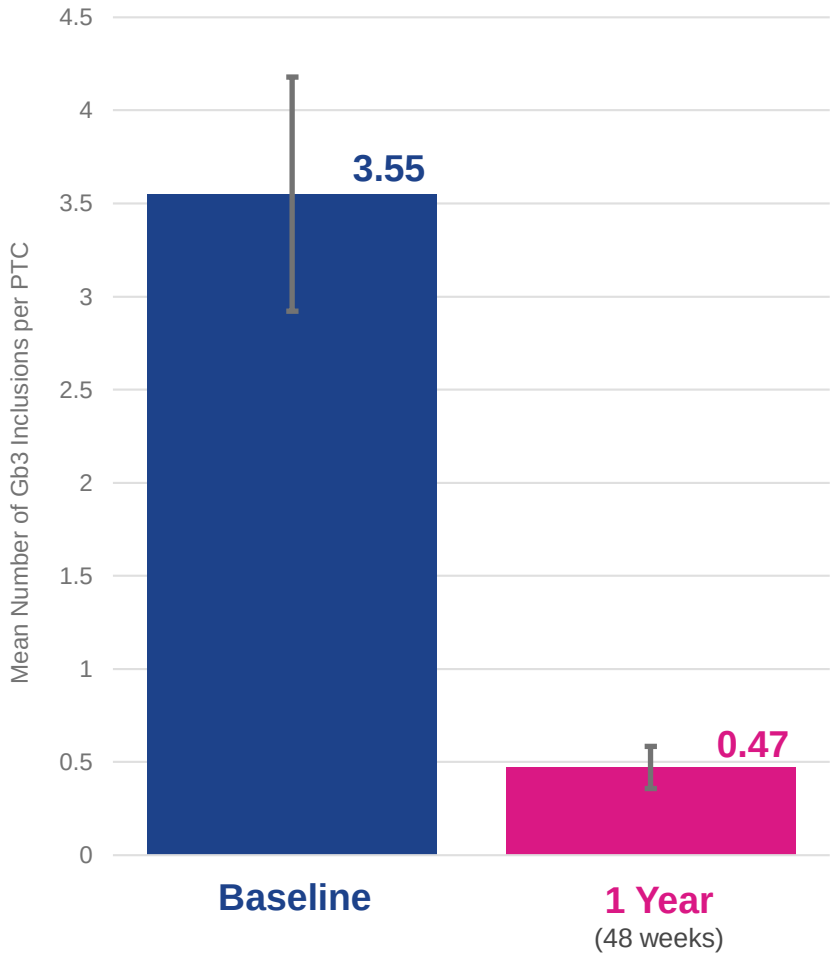
Positive trends at six months, including kidney function measures

- eGFR and serum creatinine measures trending positively at 6 months
- Pill burden remains significantly lower than at baseline

Appendix

Patient 1: 87% substrate reduction in kidney biopsy at 1 year

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
PTC: Peritubular Capillary; Gb3: Globotriaosylceramide; GL-3: Globotriaosylceramide; KIC: Kidney Interstitial Capillary

New collaborations advancing leadership in lentiviral gene therapy

Fully Automated Bu-TDM Immunoassay

- AVROBIO and Saladax announced agreement to develop and validate Saladax's fully automated nanoparticle immunoassay kit
- Designed to work on most automated hospital analyzers
- Regular technician, 24/7 analysis possible with results anticipated in minutes using only microLs of blood
- Designed to analyze 10-100s samples per machine/hour
- Expected to eliminate Bu degradation errors as assay conducted in real-time at the point of care
- Scalable

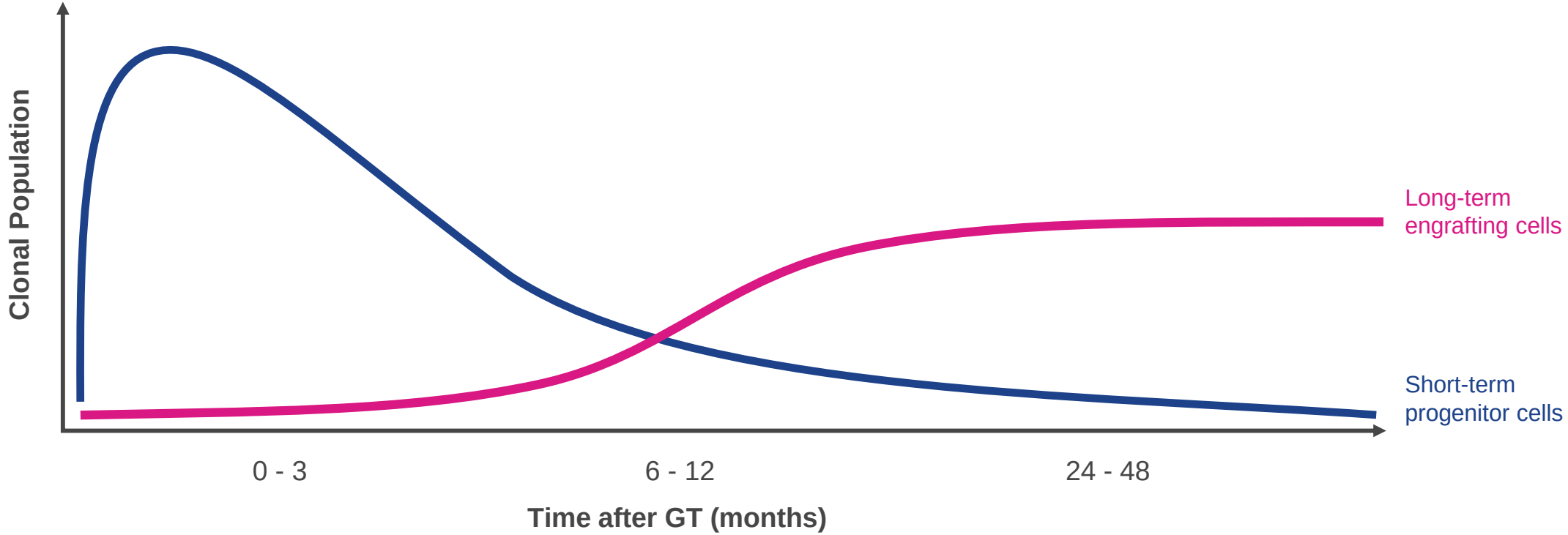
Antibody-Drug Conjugate

- AVROBIO and Magenta announced research & clinical collaboration agreement to evaluate Magenta's preclinical CD117-targeted antibody conjugate to amanitin (MGTA-117) in conjunction with AVROBIO investigational gene therapies
- Designed to deplete only hematopoietic stem and progenitor cells
- Has shown promising data in non-human primates
- MGTA-117 currently in IND-enabling studies
- Each party retains commercial rights to its own programs

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



Source: Biasco L et al, Cell Stem Cell, 2016