Company Presentation

July 2020

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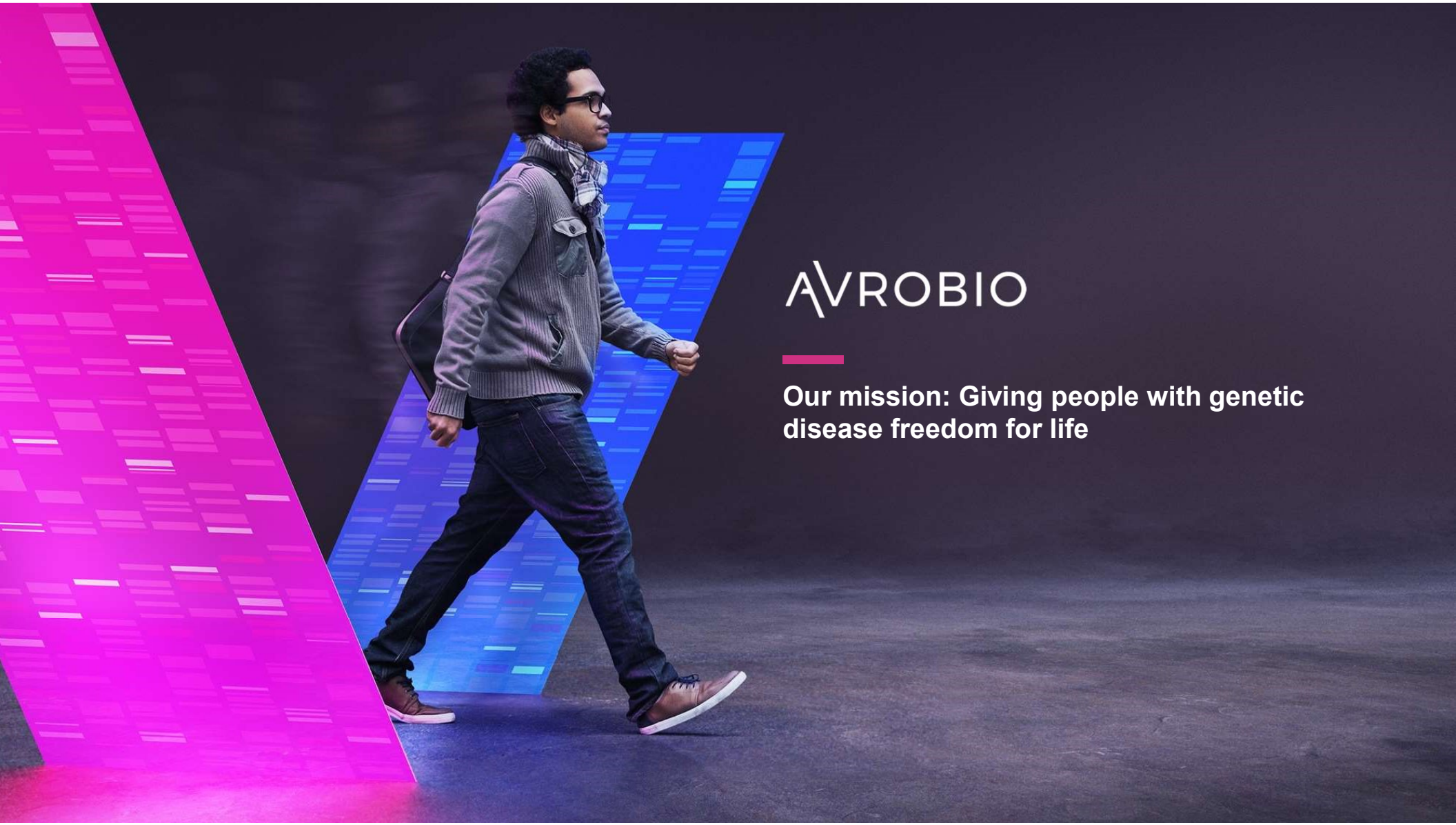

AVROBIO
Our mission: Giving people with genetic disease freedom for life

Multiple programs in the clinic

12 patients dosed to date across three clinical trials

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

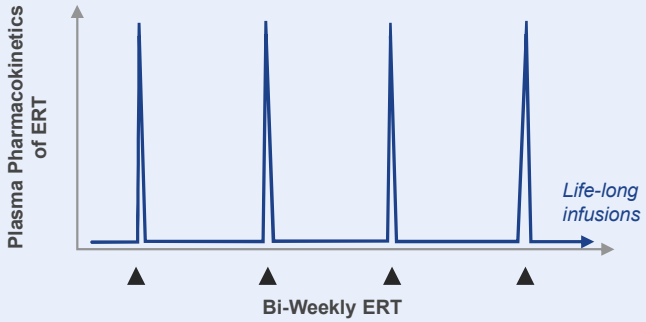
Addressing multi-billion dollar market opportunity

CURRENT STANDARD OF CARE COSTS

Disease	Est. Cost Per Patient Per Year	Approx. 2019 Net Sales	Selected Companies
Fabry	$320k	$1.4B	SANOFI GENZYME, Takeda Shire
Gaucher	$250k-400k	$1.4B	SANOFI GENZYME, Takeda Shire
Pompe	$500k	$1.0B	SANOFI GENZYME
Cystinosis	$625k-700k*	$0.2B	HORIZON PHARMA, Mylan, RECORDATI

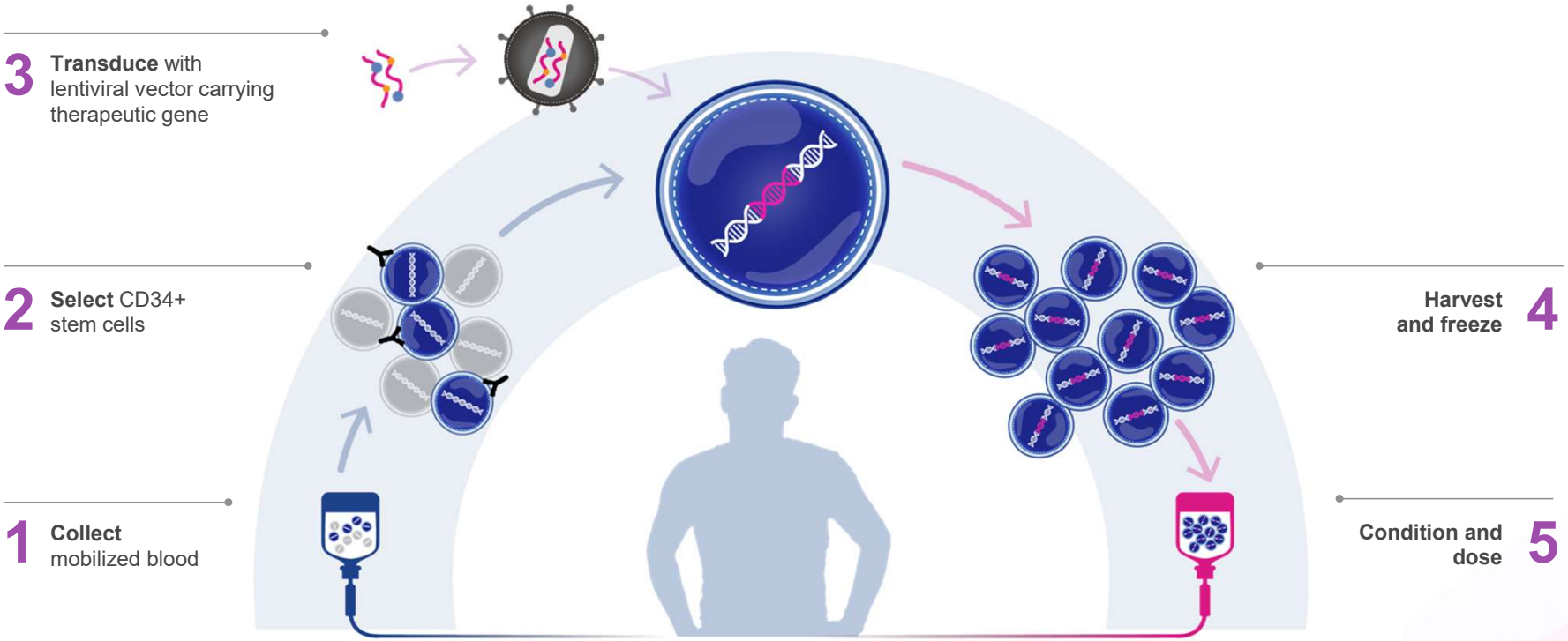
Sources: Rombach S et al, Orphanet J Rare Dis, 2013; van Dussen L et al, Orphanet J Rare Dis, 2014; WAC pricing from Redbook; 2019 Net Sales from company annual and other reports
** for Horizon's Procysbi oral therapy (delayed release cysteamine bitartrate)*
Note: Shire acquired by Takeda in 2019

Lifelong treatments vs. potential single-dose therapy

	DISEASE PROGRESSION CONTINUES	DISEASE PROGRESSION COULD HALT OR REVERSE
	Enzyme Replacement Therapy (ERT) Temporary bolus of enzyme, not curative	**AVROBIO Gene Therapy** Designed for 24/7 expression of protein, curative potential
		
Enzyme or protein level	Transient, intermittent elevation	Long-term, continuous elevation
Treatment burden	Bi-weekly IV infusions	Single IV infusion

ERT: Enzyme Replacement Therapy; IV: Intravenous; HSC: Hematopoietic Stem Cells

Established *ex vivo* lentiviral approach



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

July 2019 data presented, unless otherwise specified
** Sponsored by FACTs team (Fabry Disease Clinical Research and Therapeutics) in Canada*

Fabry FAB-201 Patient Characteristics

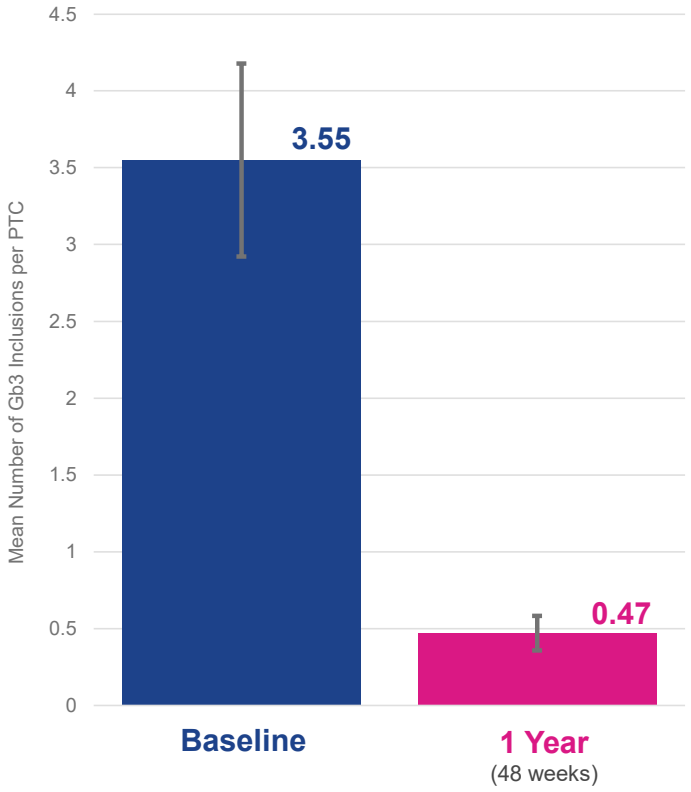
Treatment-naïve Fabry patients

	PATIENT 1	PATIENT 2	PATIENT 3	PATIENT 4
Age of symptom onset / diagnosis	10 / 19 years	36 / 37 years	13 / 13 years	9 / 9 years
Age dosed with AVR-RD-01	21 years	46 years	40 years	26 years
Mutation	c.1021G>A (p.E341K)	c.644A>G (p.N215S)	c.639+1G>T	c.833dupA
Primary disease signs and symptoms	• Kidney disease • Chronic pain • GI symptoms • Decreased cold sensation	• Cardiac disease • Peripheral neuropathy • Chronic pain • Increased tiredness • GI symptoms • Intermittent tinnitus • Mild high frequency hearing loss • Raynaud's syndrome	• Kidney disease • GI symptoms • Peripheral neuropathy • Bilateral deafness • Tinnitus • Peripheral edema • Decreased cold sensation	• Chronic pain • Peripheral neuropathy • Neuropathic shuffling gait • Lethargy • Temperature intolerance • Tinnitus • Hearing loss • GI symptoms
Leukocyte AGA enzyme activity at baseline (nmol/hr/mg protein)	0.10*	2.38**	0.58**	0.46**
Plasma lyso-Gb3 at baseline (nM)	202***	8***	147***	92***
Comment	IgA deposits in kidney biopsy	Cardiac variant, not a classic Fabry male		

** Mayo Lab, ref range ≥23.1 nmol/hr/mg*
*** Rupar Lab, ref range 24-56 nmol/hr/mg*
**** Reference value ≤ 2.4 nM*
AGA: α-galactosidase A; Lyso-Gb3: Globotriaosylsphingosine; GI: Gastrointestinal; IgA: Immunoglobulin-A

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

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

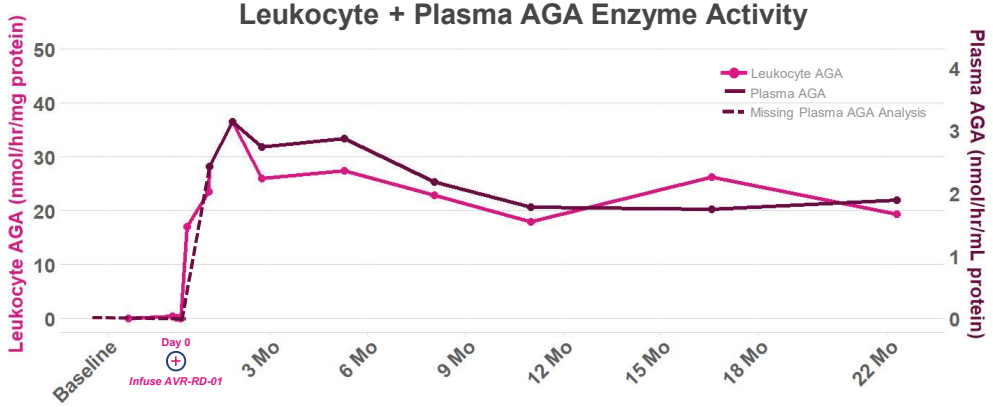


- *Unpaired t-test for difference between n=55 PTCs at baseline vs. n=101 PTCs at 1 year; p < 0.0001*
- *Error bar represents the standard deviation*

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

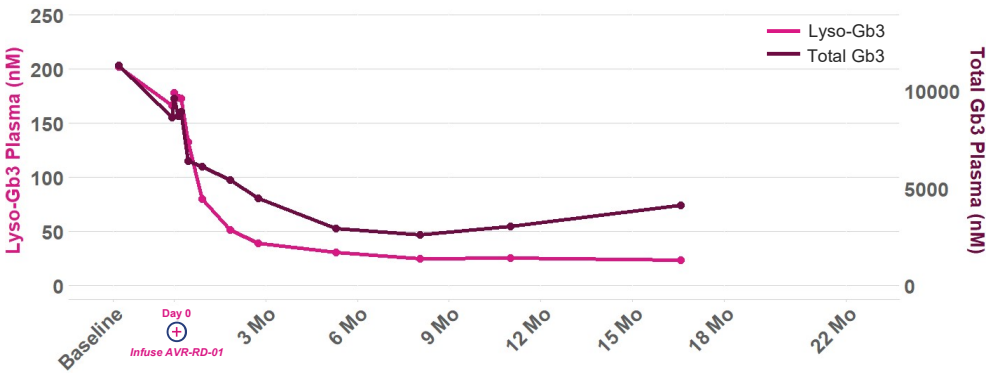
Patient 1: Multiple data trends sustained up to 22 months

Leukocyte + Plasma AGA Enzyme Activity



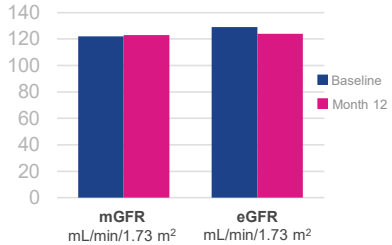
*Lab A: Mayo Clinic Laboratories; Lab B: Rupar Laboratory; Lab A Reference Range: >23.1 nmol/hr/mg; Lab B Reference Range: 24–56 nmol/hr/mg
†Reference Range: 5.1–9.2 nmol/hr/mL; AGA: α-galactosidase A

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

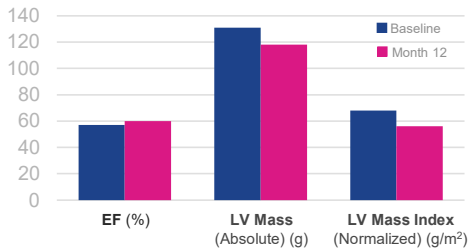
KIDNEY FUNCTION
remains within normal range at 12 mos.



Normal Range mGFR/eGFR	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

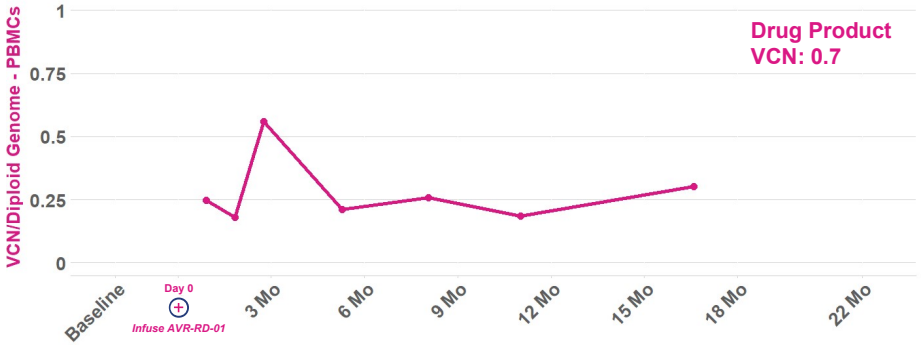
CARDIAC FUNCTION
remains within normal range at 12 mos.



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

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

Vector Copy Number (VCN)

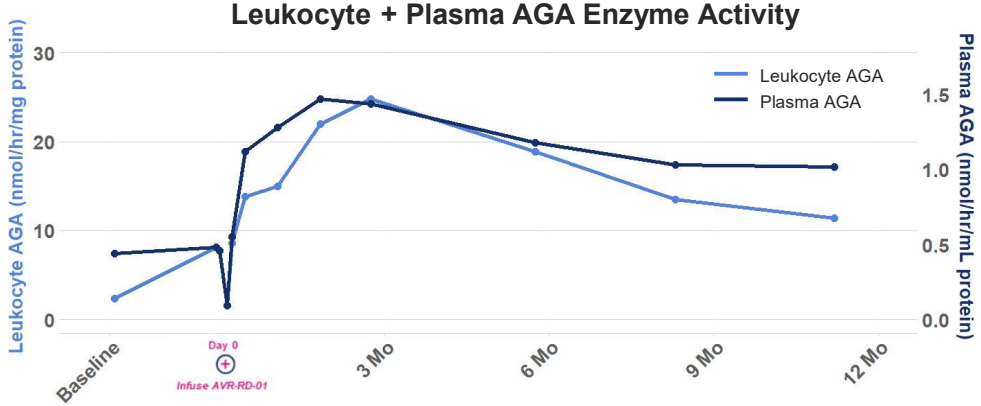


VCN: Vector Copy Number; PBMCs: Peripheral Blood Mononuclear Cells

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)

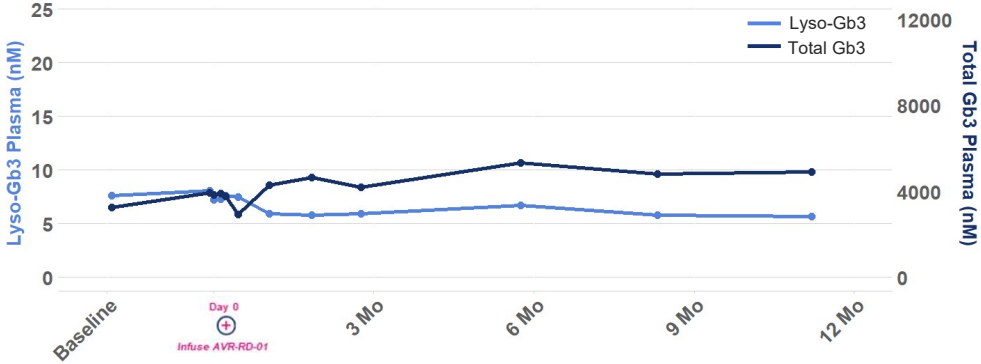
Patient 2: Multiple data trends sustained up to 1 year*

Leukocyte + Plasma AGA Enzyme Activity



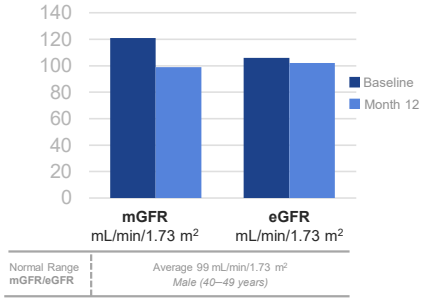
*Data from Rupar Laboratory; Reference Range: 24–56 nmol/hr/mg; †Reference Range: 5.1–9.2 nmol/hr/mL; AGA: α-galactosidase A

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

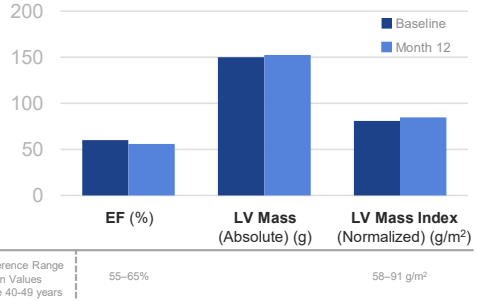
KIDNEY FUNCTION *remains within normal range*



	mGFR mL/min/1.73 m²	eGFR mL/min/1.73 m²
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*



	EF (%)	LV Mass (Absolute) (g)	LV Mass Index (Normalized) (g/m²)
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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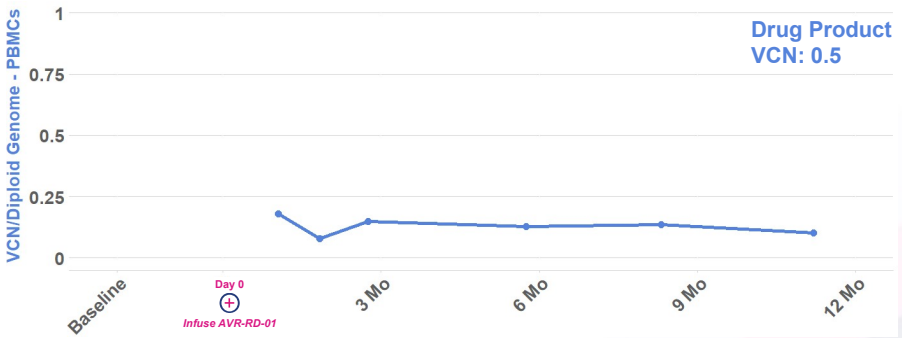

VCN: Vector Copy Number; PBMCs: Peripheral Blood Mononuclear Cells

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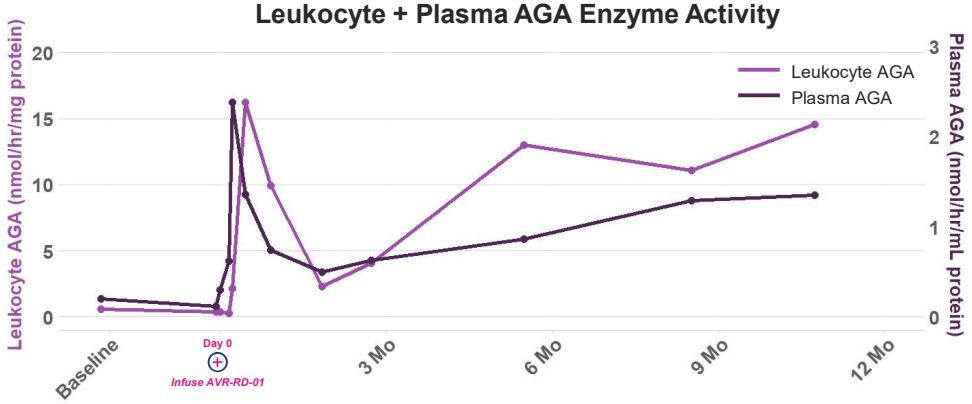
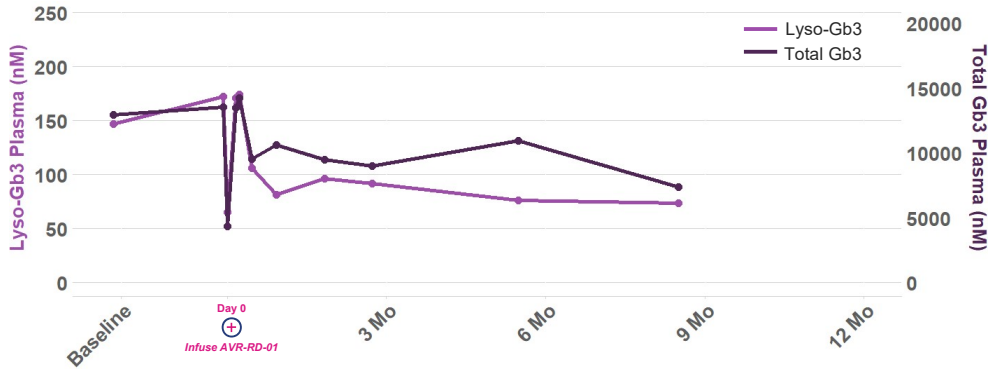
Patient 3: Data up to 1 year* suggest trend towards durable engraftment

Leukocyte + Plasma AGA Enzyme Activity



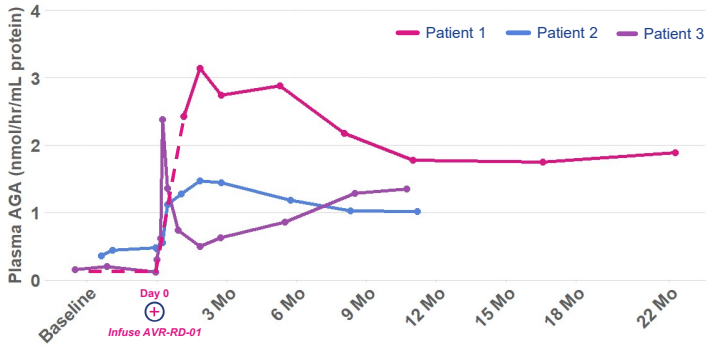
*Data from Rupar Laboratory; Reference Range: 24–56 nmol/hr/mg; †Reference Range: 5.1–9.2 nmol/hr/mL; AGA: α-galactosidase A

Plasma Lyso-Gb3 and Total Gb3



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

Plasma AGA Enzyme Activity



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

Skin Biopsy Score (Patient 3)	
Baseline	2
6 months	2

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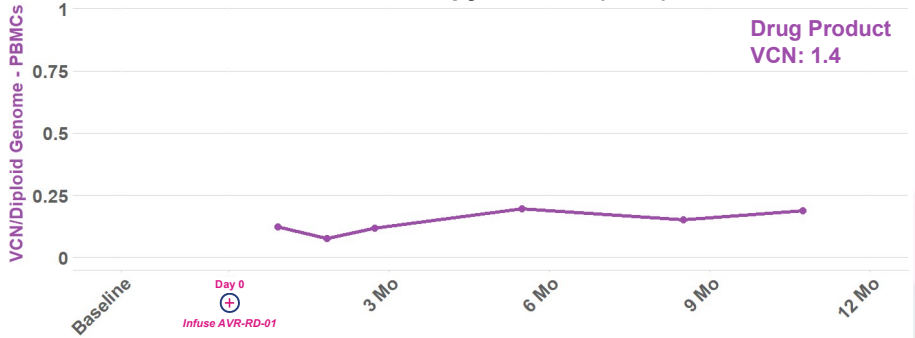
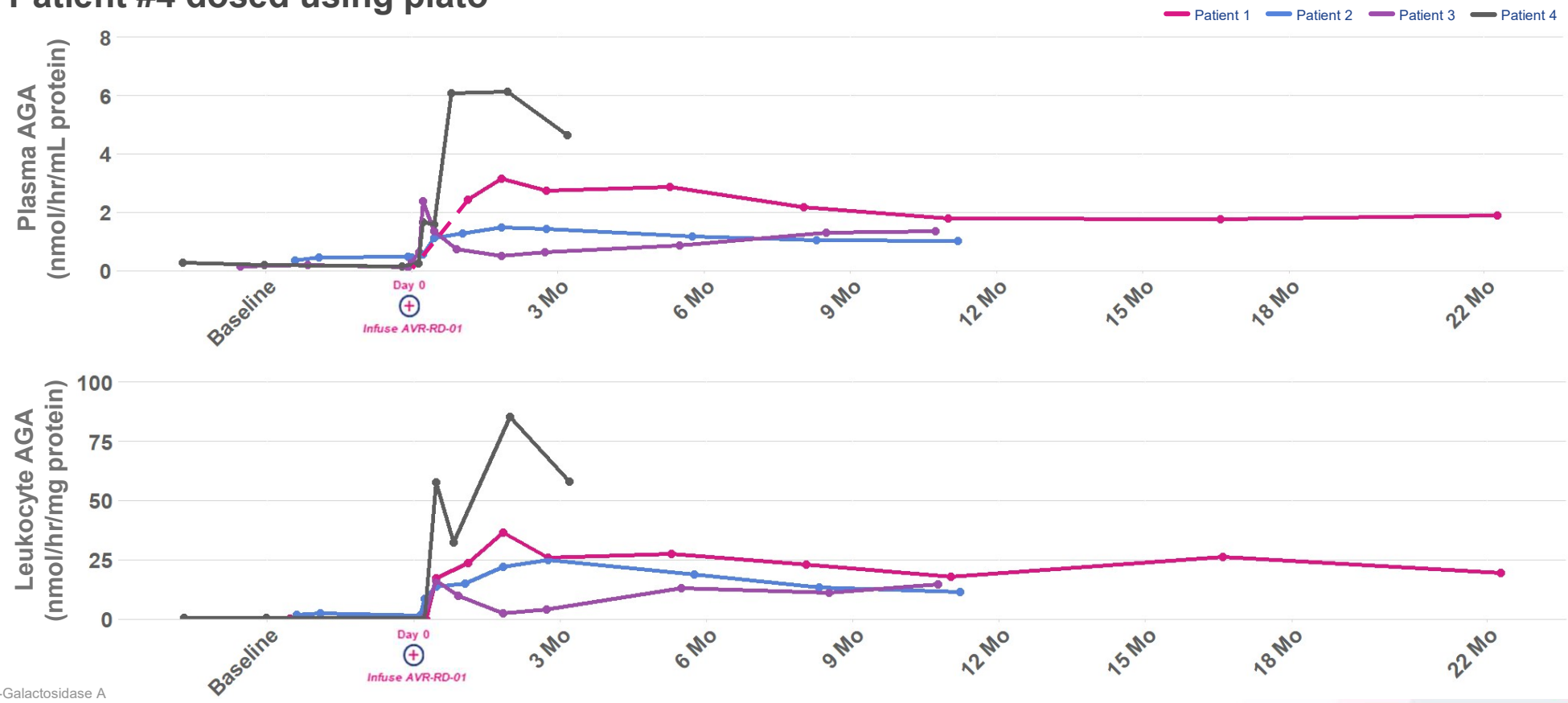


VCN: Vector Copy Number; PBMCs: Peripheral Blood Mononuclear Cells

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*

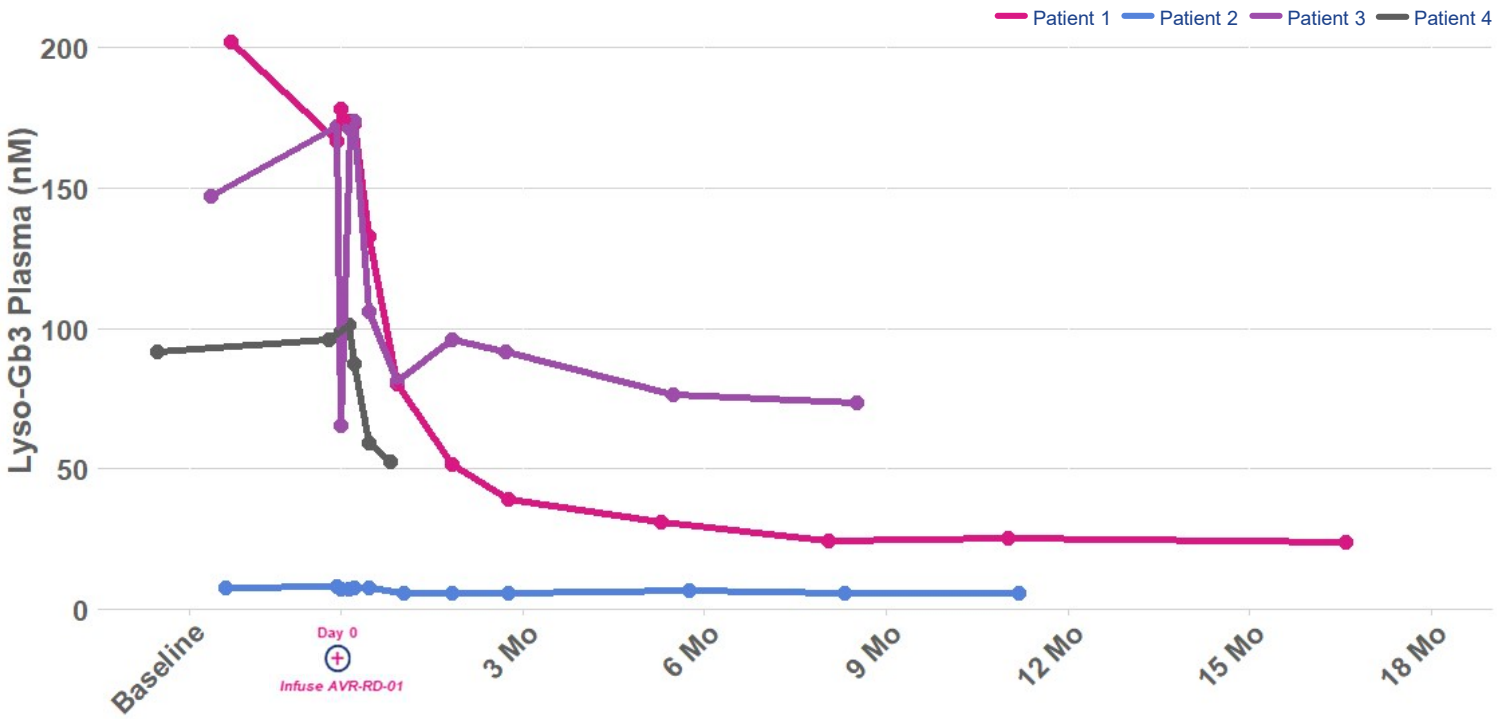
Patients 1-4: Plasma and leukocyte enzyme activity sustained up to 22 months

Patient #4 dosed using plato®



AGA: α-Galactosidase A

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

Fabry Phase 1 Patient Characteristics

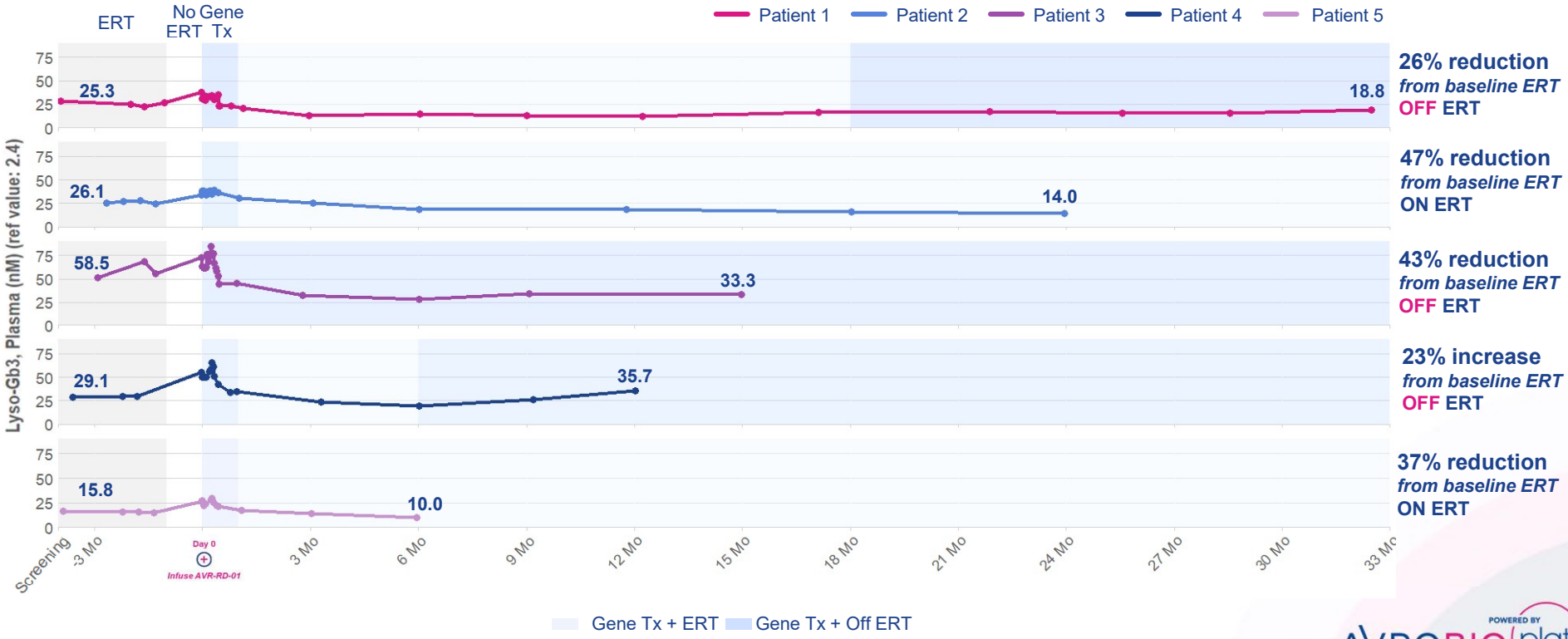
ERT-Treated Fabry Patients

	PATIENT 1	PATIENT 2	PATIENT 3	PATIENT 4	PATIENT 5
Age of symptom onset / diagnosis	18 / 37 years	9 / 29 years	10 / 0 years	7 / 4 years	10 / 14 years
Years on ERT	11 years	6 years	4 years	11 years	2 years
Age dosed with AVR-RD-01	48 years	39 years	40 years	37 years	30 years
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	• Kidney disease • Cardiac disease • GI pain • GI diarrhea • Angiokeratoma • Insomnia	• Kidney disease • Cardiomyopathy • Hypohidrosis • Corneal verticillata • Peripheral neuropathy • GI symptoms • Angiokeratoma • Lymphedema • Acroparesthesia	• Cardiac Disease • Tinnitus • Headaches • Dizziness • Acroparesthesia	• Cardiac Disease • Hypohidrosis • Tinnitus • Corneal verticillata • Angiokeratoma • GI symptoms	• Kidney disease • Hypertension • Hypohidrosis • Tinnitus • Migraines • Impaired hearing • Angiokeratoma • Sleep apnea • Asthma • Depression
Leukocyte AGA activity at baseline (nmol/hr/mg protein)	2.1*	1.1*	0.6*	2.2*	1.0*
Plasma lyso-Gb3 at baseline (nM)	25**	26**	59**	29**	16**
ERT discontinuation status	18 months after gene therapy dose		Did not resume ERT after gene therapy dose	6 months after gene therapy dose	

** Rupar Lab, ref range 24-56 nmol/hr/mg*
*** Reference value ≤ 2.4 nM*
Note: AGA: α-galactosidase A; ERT: Enzyme Replacement Therapy; GI: Gastrointestinal; Lyso-Gb3: Globotriaosylsphingosine

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

All patients who have discontinued ERT remain off ERT*

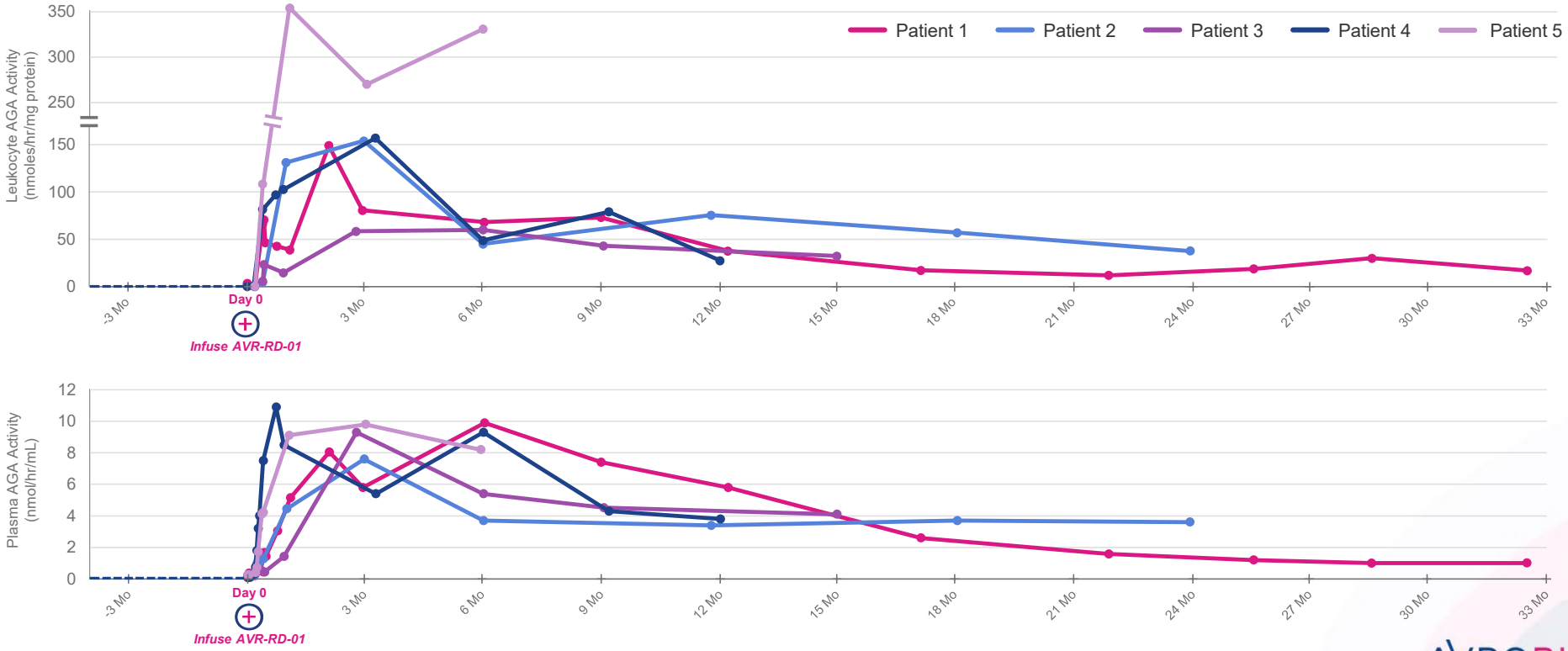


* As of April 27, 2020

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

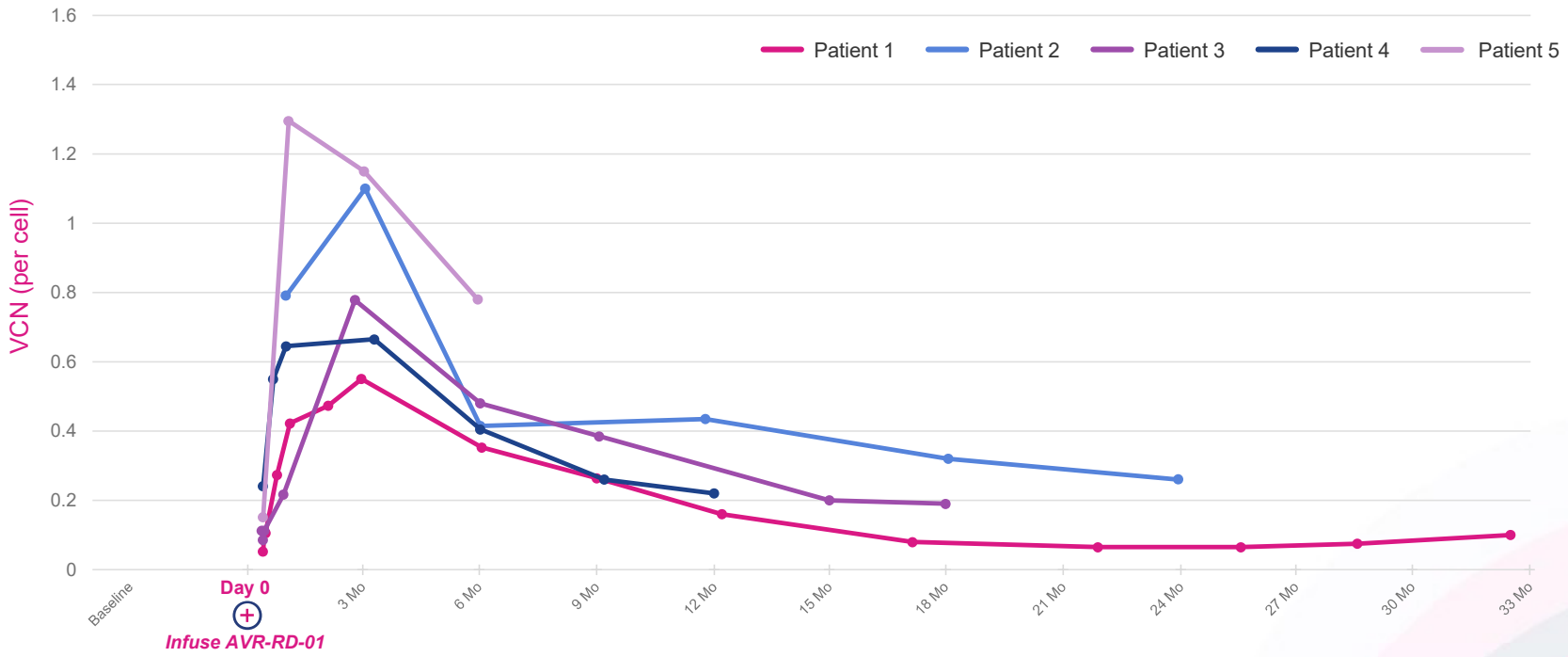


AGA: α-Galactosidase A

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

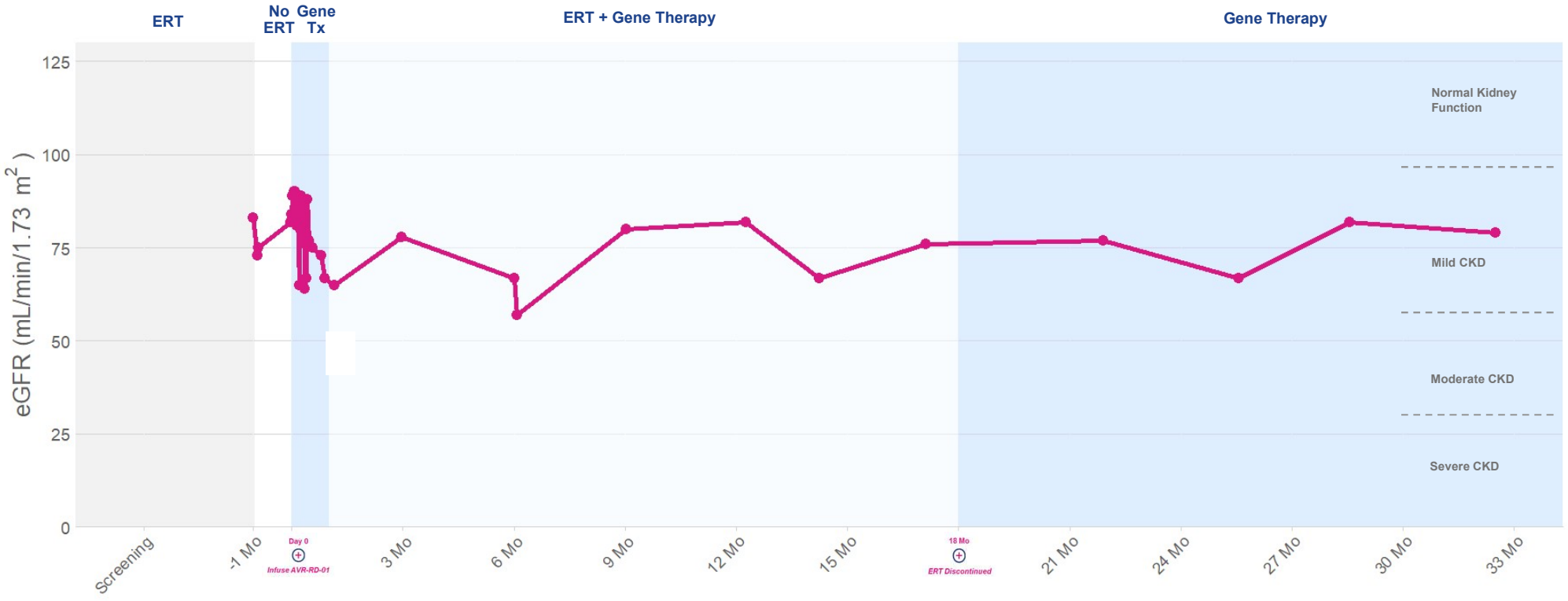
4 patients with 1+ years data

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)

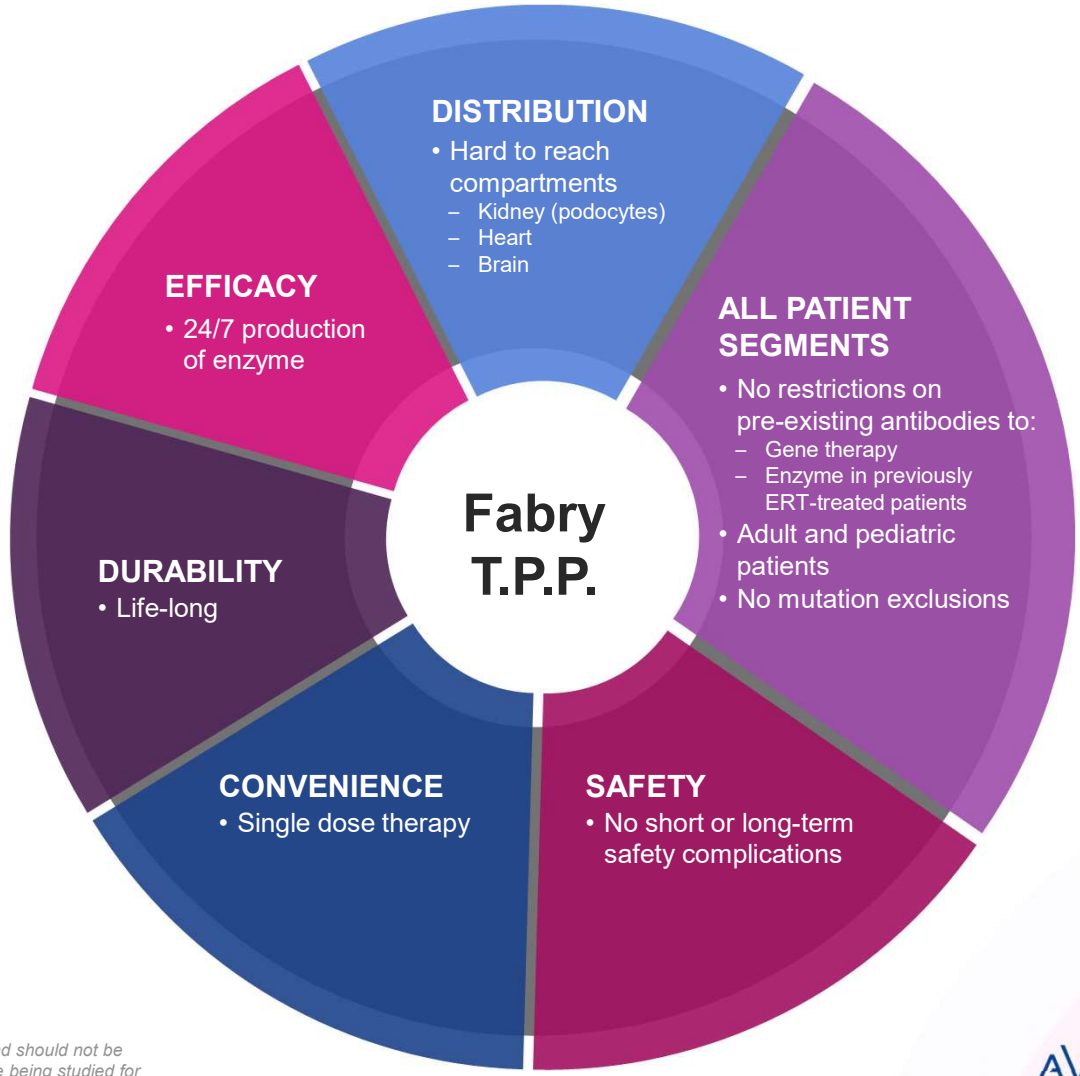
Anti-AGA antibodies

- 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

Fabry disease target product profile (T.P.P.)

Potential attributes intended to support first-line use



ERT: Enzyme Replacement Therapy
Note: Potential attributes represent desired target product profile, and are not intended, and should not be interpreted, to be attributes of AVROBIO's current investigational gene therapies, which are being studied for safety and efficacy and have not been approved by the FDA or any other regulatory body.

Building commercial capabilities

44+ product launches, including 1 gene therapy

Holly May
Chief Commercial Officer

- Led commercial teams for LSDs at SanofiGenzyme
- Head of Commercial at SOBI, a rare disease company

SANOFI GENZYME
sobi
Pioneer in Rare Diseases

Jose Gomez
SVP, Global Market Access & Value

- Led global strategic marketing and global market access functions at AveXis
- Led market access, global strategic pricing and reimbursement functions for LSDs at Shire

Shire avexis

Sean Ring
VP, Head of Commercial Operations

- Extensive orphan drug commercial strategy and launch expertise
- Built out market development and commercial ops at Editas, Zafgen, Cubist, Shire, Biogen

Shire editas MEDICINE

Ramesh Arjunji
VP, Global Health Economics and Outcomes Research / Value Demonstration

- Led value demonstration for insurers globally at AveXis
- Significant access and reimbursement responsibilities at leading global biotech companies

avexis Biogen

POWERED BY AVROBIO plato

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

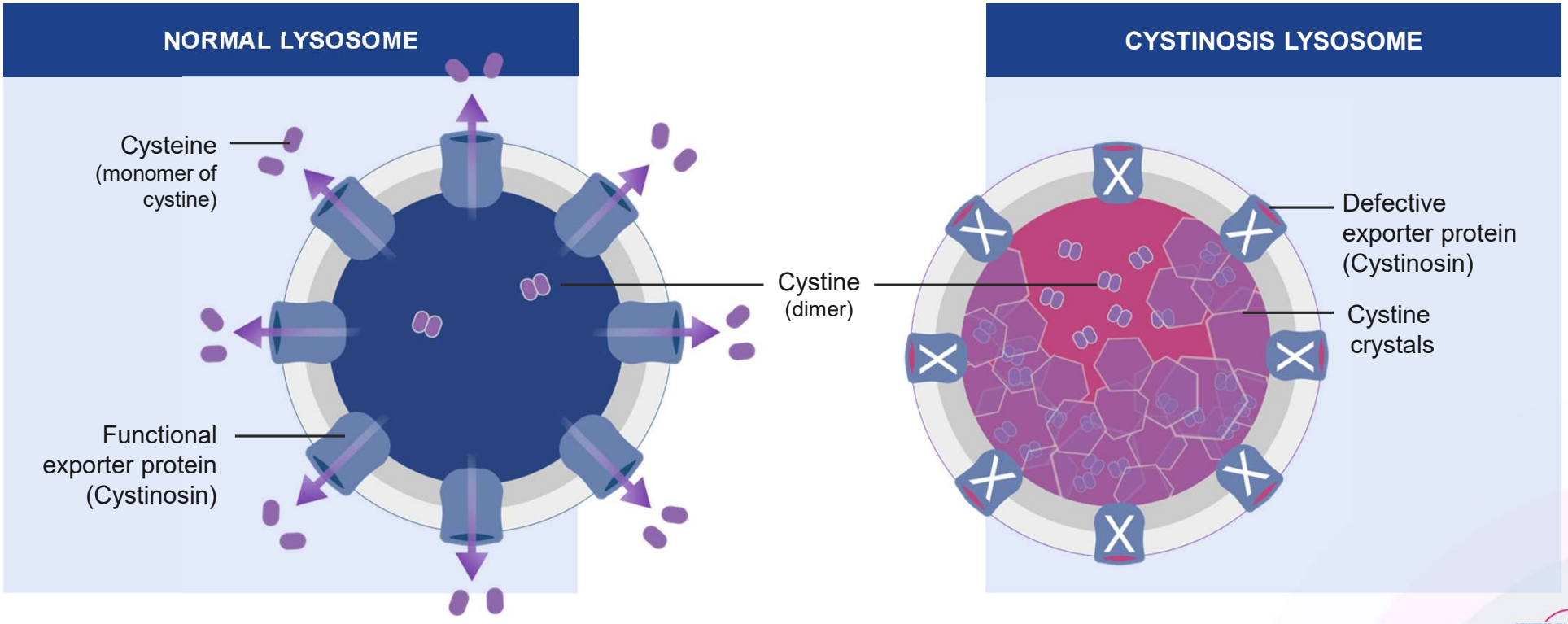
CNS complications
Unmet needs: myopathy, hypotonia, tremors, difficulty swallowing, neurodevelopmental issues (speech and walking delay and cognitive impairment)

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

Cystinosis caused by defective gene that encodes cystinosin, an exporter protein

Cystine crystals build up in lysosomes causing tissue and organ damage



Source: Cherqui et al, Nat Rev Nephrol. 2017

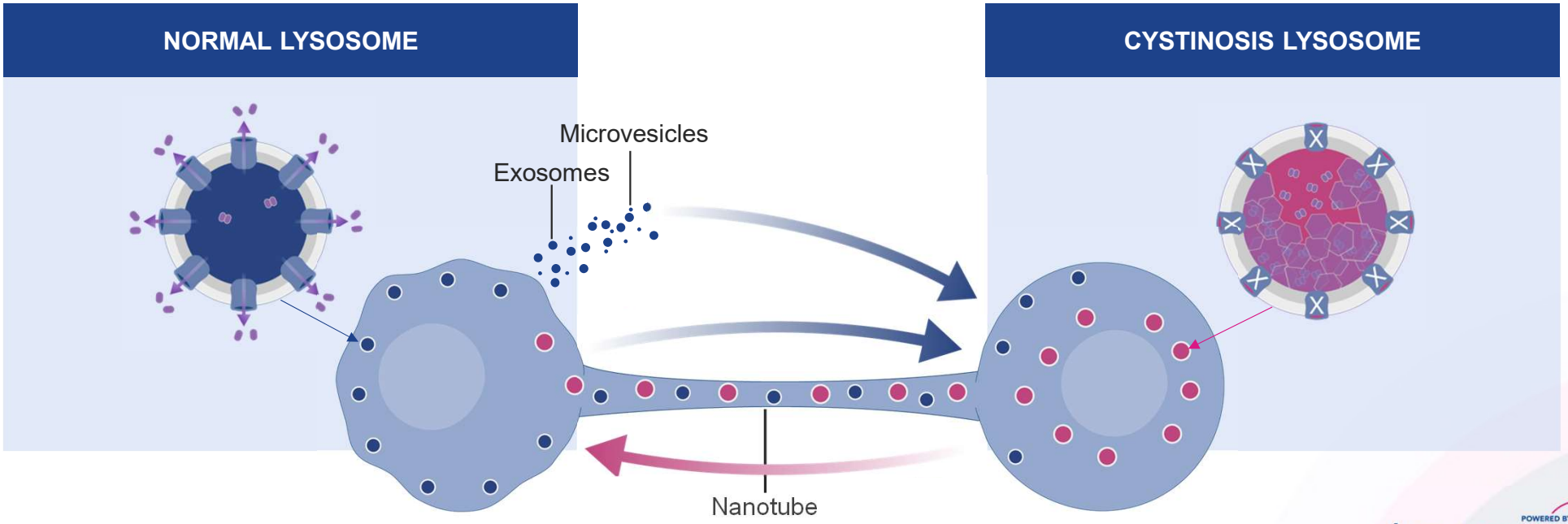
Drug product-derived macrophages restore normal cystine recycling

Mechanisms of action

Macrophages with CTNS transgene restore cystine recycling to CTNS^{-ve} cells via:

1. Tunneling nanotubes – transfer of corrected lysosomes, cystinosin, CTNS mRNA
2. Exosomes / Microvesicles – transfer of cystinosin, CTNS mRNA

Net result: Corrected lysosomes in cells throughout the body



Sources: Naphade, Stem Cells, 2015. Harrison, Molecular Therapy, 2013.
CTNS: cystinosin, lysosomal cystine transporter; mRNA: Messenger Ribonucleic Acid

Allogeneic transplant demonstrated stabilized renal function, corrected polyuria and improved photophobia

Allogenic HSC Transplant

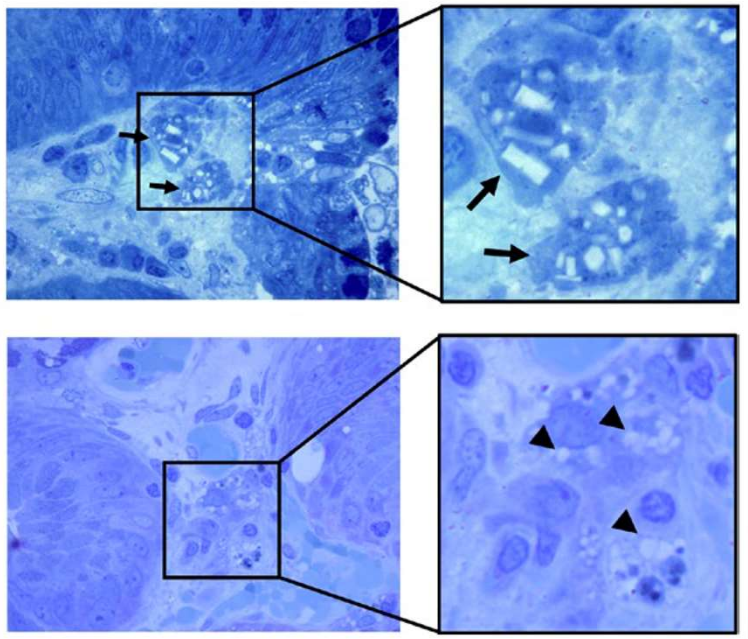
University Hospital Leuven

- 16 year old male
- Diagnosed at 2.7 years old, started on cysteamine
- Age 15 years – cysteamine toxicity
- Age 16 years – fully matched HLA transplant
- Acute GvHD
- **First few months**
  - **Kidney function stabilized**
  - **Polyuria resolved**
- **6 months**
  - **Photophobia score reduced from 5 (unable to open eyes even inside dark room) to 0 (no photophobia)**

Cystine crystal reduction (31%) in macrophages in gastric mucosa at 30 months post transplant

BEFORE TRANSPLANT

30 MONTHS POST TRANSPLANT



Arrows/arrowheads point to tissue macrophages

Elmonem M A et al, Am. J. Transplant, 2018; HSC: Hematopoietic Stem Cell; HLA: Human Leukocyte Antigen; GvHD: Graft vs Host Disease

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

Two patients 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

Phase 1/2 Cystinosis

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

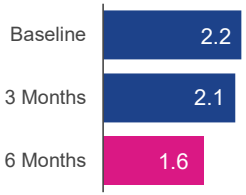
Note: Safety database cut as of January 27, 2020 for first patient dosed in the trial
AE: Adverse Event; SAE: Serious Adverse Event

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*

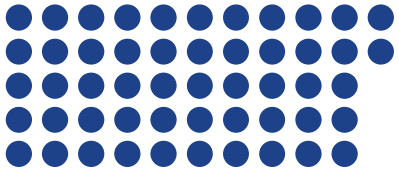
Patient 1: Reduced treatment burden at 6 months

Number of Medications and Supplements

(max per day)

Before Gene Therapy

ON Cysteamine



52

After Gene Therapy

(at 6 months post-gene therapy)

OFF Cysteamine

20

NOTE: Investigational gene therapy

Gaucher Disease

AVR-RD-02

Goals for gene therapy in **Gaucher Type 1 Disease**

UNMET NEEDS:

Bone-related manifestations
Unmet needs: bone pain, avascular necrosis, bone crisis, osteoporosis, fractures, joint destruction, skeletal abnormalities

Hemoglobin levels and platelet counts
Unmet needs: anemia, thrombocytopenia, easy bruising, bleeding

Hepatosplenomegaly
Unmet needs: enlarged liver, enlarged spleen

CNS complications
Unmet needs: Increased risk of GBA-Parkinson's disease

Everyday burden of illness, and life expectancy
Unmet needs: fatigue, pain, lung disease, biweekly infusions, shortened lifespan

Sources: Grabowski G et al, Online Metabolic and Molecular Bases of Inherited Disease, 2018; Weinreb N et al, AJH, 2008; Pastores G et al, Semin Hematol, 2004
CNS: Central Nervous System; GBA: gene coding for glucocerebrosidase

Long-term follow-up study highlights significant unmet need in Gaucher Type 1

Despite standard-of-care ERT, **disease progression** continues and **unmet need** remains.

Incomplete therapeutic response is common:

- **60%** of patients failed to achieve at least one of the six therapeutic goals evaluated after 4+ years of ERT[1]
- A clinically significant percentage of patients continue to exhibit **bone pain, organomegaly and cytopenia** after 10 years of ERT[2]
- **25%** of patients continue to suffer from physical limitations after two years of ERT, primarily due to bone disease[3]

Persistence after **10 years ERT†**	**Non-splenectomized** Patients	**Splenectomized** Patients
Anemia	12.4%	8.8%
Thrombocytopenia*	22.7%	0.7%
Splenomegaly*	38.3%	N/A
Hepatomegaly*	14.3%	18.8%
Bone Pain	42.9%	62.5%
Bone Crisis	7.4%	16.7%

* Higher persistence rates observed when more severe manifestations were present at baseline

† Persistence refers to the presence of anemia, bone pain, bone crisis, or at least moderate thrombocytopenia, splenomegaly, or hepatomegaly, present after 10 years of ERT among those with baseline involvement of these parameters (from a registry of 757 GD1 patients; Weinreb et al., 2013).

Following 10 years of treatment, ~26% of patients were receiving between 45-150 U/kg EOW, and 96% of these individuals were receiving doses between 45-90 U/kg EOW.

Sources: *[1]Weinreb N et al. Amer J Hematol, 2008; [2]Weinreb N et al. J Inherit Metab Dis, 2013; [3]Giraldo P et al. Qual Life Res, 2005.*

GD1: Gaucher Disease Type 1; SOC: Standard of Care; ERT: Enzyme Replacement Therapy; EOW: Every Other Week

GuardOne: Phase 1/2 study in Gaucher Type 1 patients

First patient dosed

PHASE 1/2
AVR-RD-02 Trial

Patients

n = 8 - 16
Type 1 Gaucher
Treatment naïve or on ERT
16 - 35 year-old
Male and Female

Key Objectives

Safety, Engraftment, Efficacy, ERT-independence

GAU-201: AVR-RD-02 Study; ERT: Enzyme Replacement Therapy

Pompe disease

AVR-RD-03

Goals for gene therapy in **Pompe Disease**

TO PREVENT OR IMPROVE:

Pulmonary function
Unmet needs: respiratory insufficiency, chronic respiratory infections, sleep apnea, artificial ventilation

Physical endurance and strength
Unmet needs: proximal myopathy, progressive muscle weakness in diaphragm, trunk and lower limbs, wheel-chair bound

CNS complications
Unmet needs: neuromuscular control, reduction in executive function, cognitive impairment

GI complications
Unmet needs: macroglossia (childhood onset), difficulty chewing and swallowing, GI symptoms, including irritable bowel-like symptoms

Everyday burden of illness, and life expectancy
Unmet needs: fatigue, hepatomegaly, independent living, biweekly infusions, shortened lifespan

Sources: *Barba-Romero M et al, Rev Neurol, 2012; Dasouki M et al, Neurol Clin, 2014; Hagemans M et al, J Neurol, 2007; Musumeci O et al, Eur J of Neurol, 2018*

Pompe lentiviral gene therapy program advancing

Integrated three-part approach

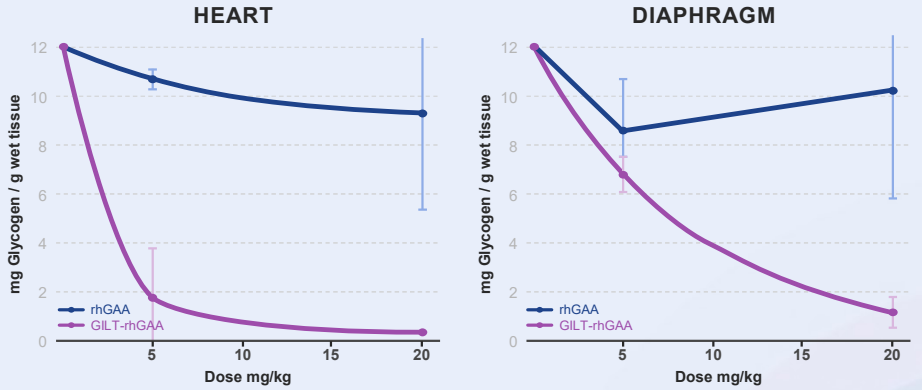
THE CHALLENGE

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

AVROBIO's APPROACH

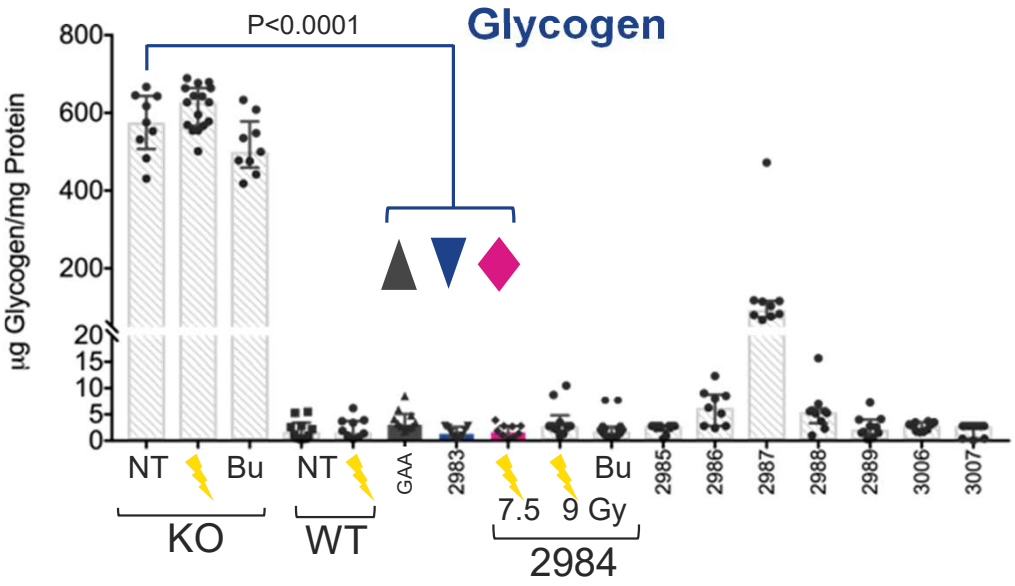
- Potent transgene promoter
- GILT uptake tag
- Bu90-TDM for CNS impact

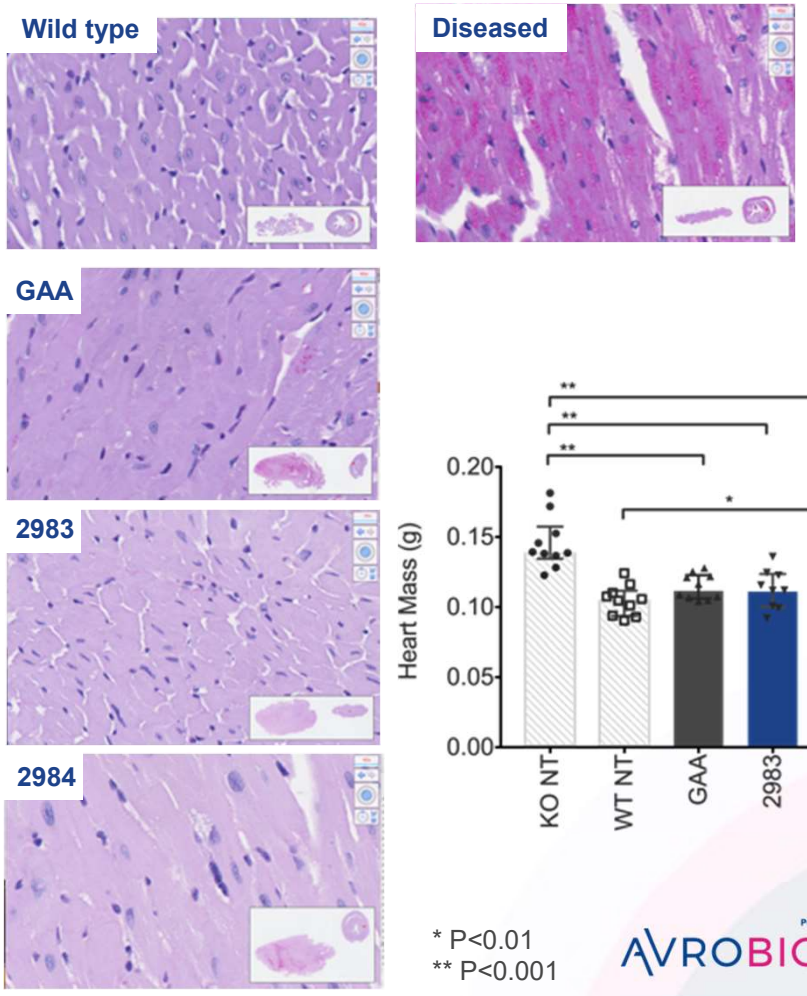
GILT-tagged Recombinant Human (rh)GAA impacts levels of stored glycogen compared to non GILT-tagged Recombinant Human (rh)GAA in a Pompe mouse model



- ***GILT:*** *Glycosylation-Independent Lysosomal Targeting*
- ***Sources:*** *Burton B et al, J Pediatr, 2017; Ausems 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; Bartelink, Lancet Haematol, 2016.*

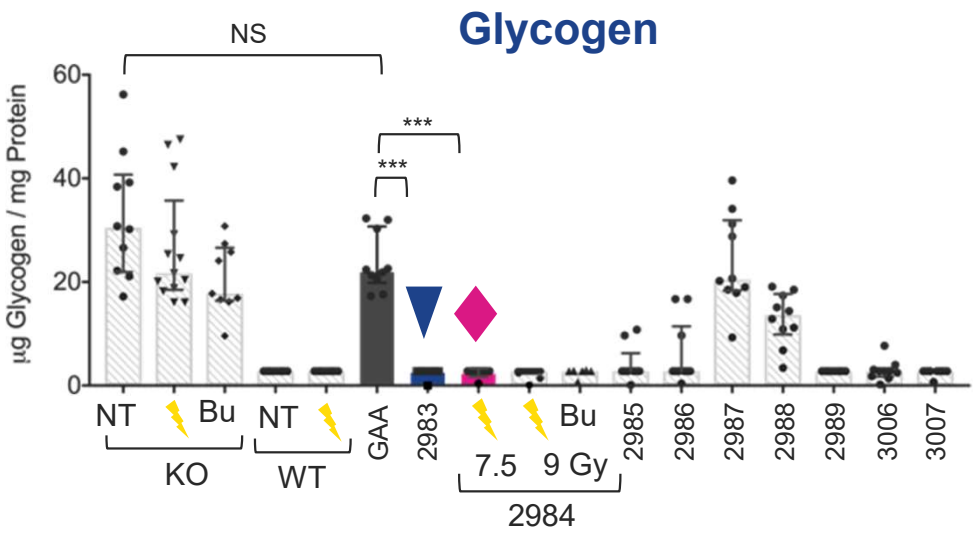
GILT and GILT mutant v1 reduce glycogen by >99% in heart

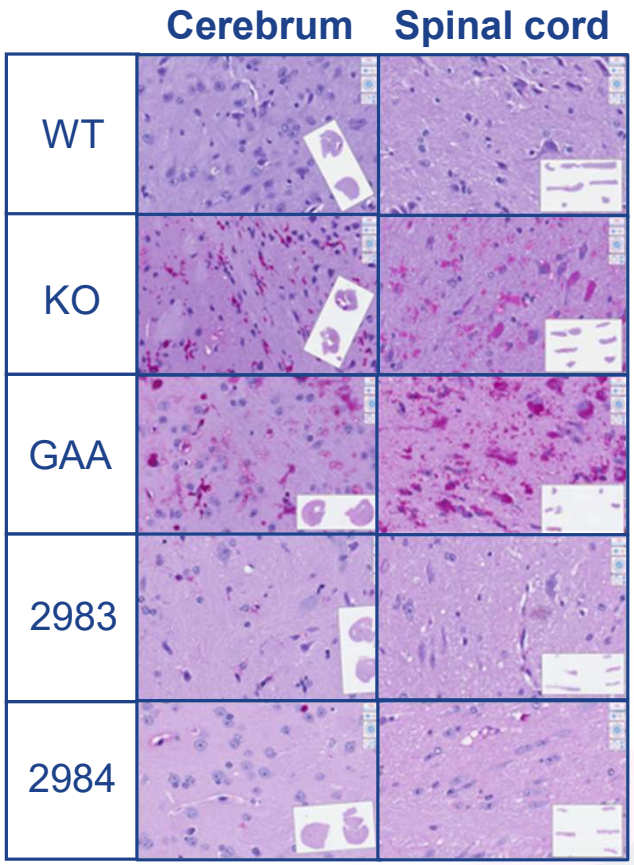




Glycogen and GILT and GILT mutant v1 similar to wildtype mice

GILT tag is essential for glycogen clearance in CNS





*** P<0.001

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)

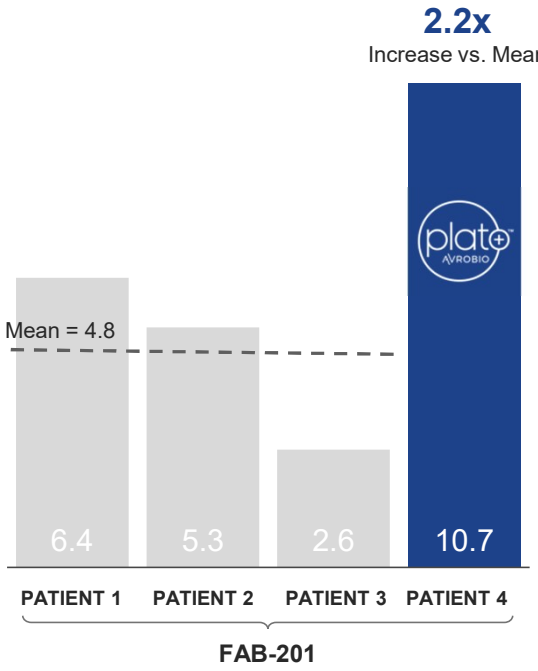
plato® UPGRADE 1

VECTOR UPGRADE:

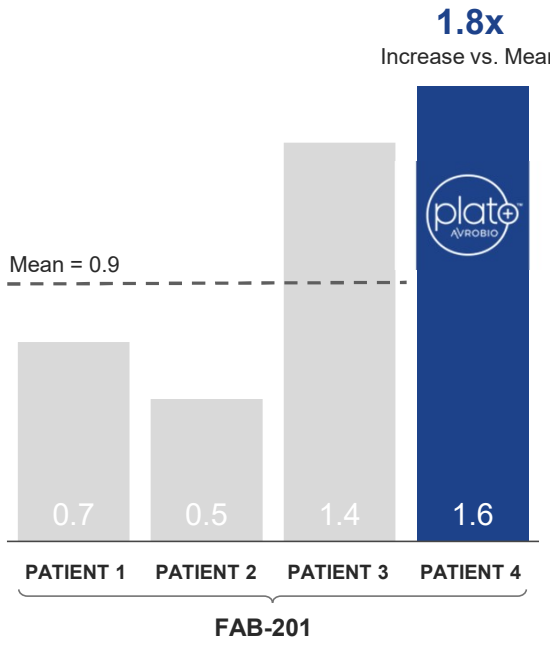
Metrics compared to academic process

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

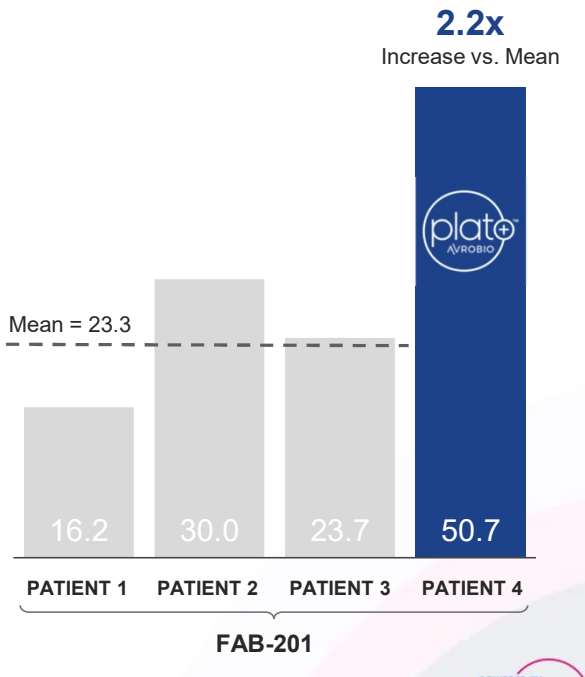
Enzyme Activity (nmol/hr/mL)



VCN (per diploid genome)



Transduction Efficiency (%)



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

POWERED BY AVROBIO plato

plato® UPGRADE 1

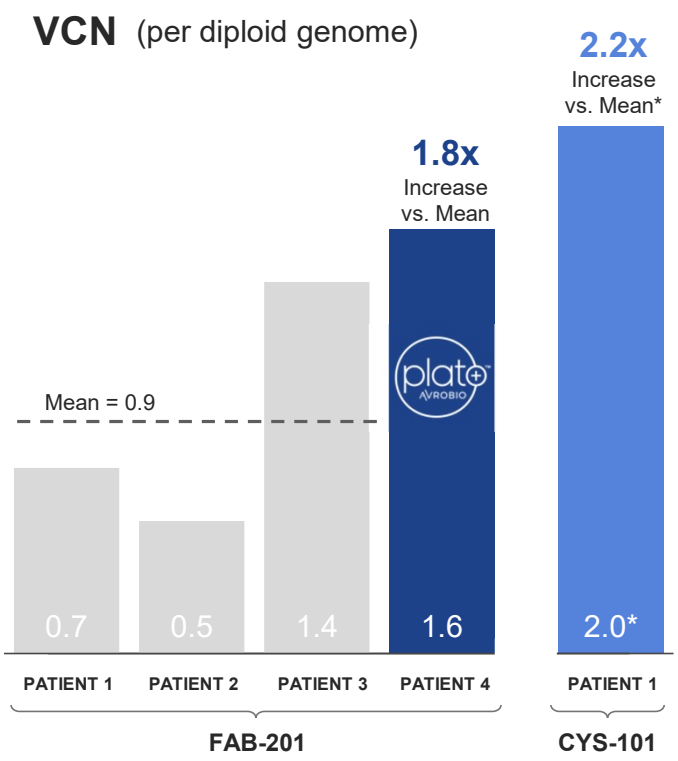
VECTOR UPGRADE:

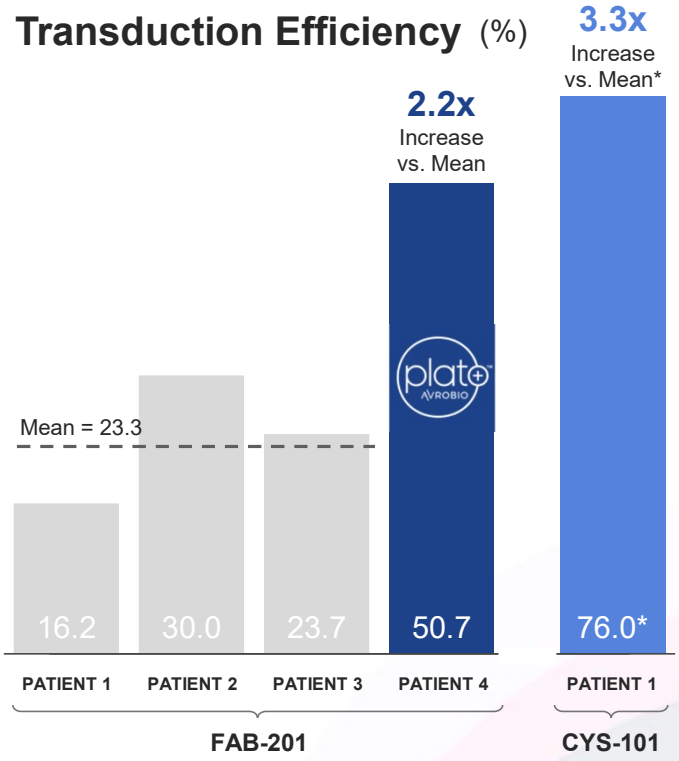
Metrics compared to academic process

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

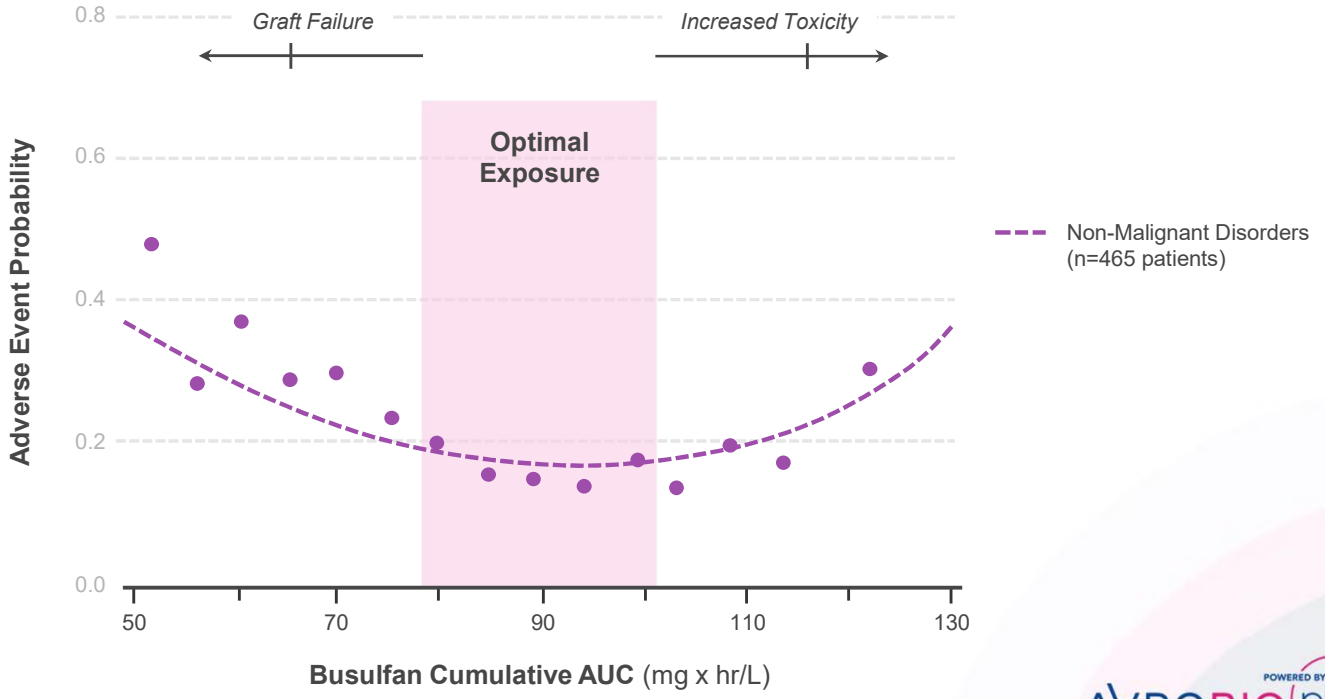
PRECISION CONDITIONING UPGRADE:

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

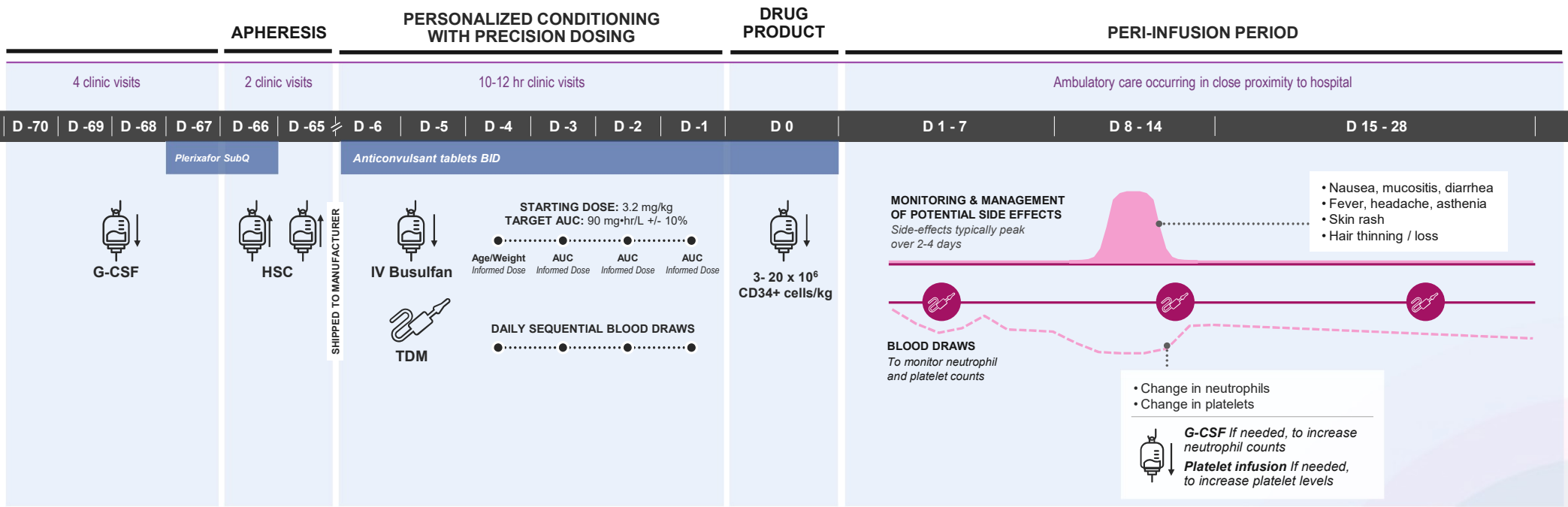


Bu: Busulfan; AUC: Area Under the Curve
Sources: Bartelink IH et al, Lancet Haematol, 2016

plato® UPGRADE 2

PRECISION CONDITIONING UPGRADE:

Precision dosing via state-of-the-art patient therapeutic drug monitoring (TDM)



G-CSF: granulocyte colony stimulating factor; PERI-INFUSION PERIOD: time from infusion to discharge; TDM: therapeutic drug monitoring; HSC: hematopoietic stem cell
Notes: Illustrative only. AEs will vary patient by patient, see busulfan label for a complete list of side-effects. Ambulatory care based on oncology setting with higher intensity conditioning

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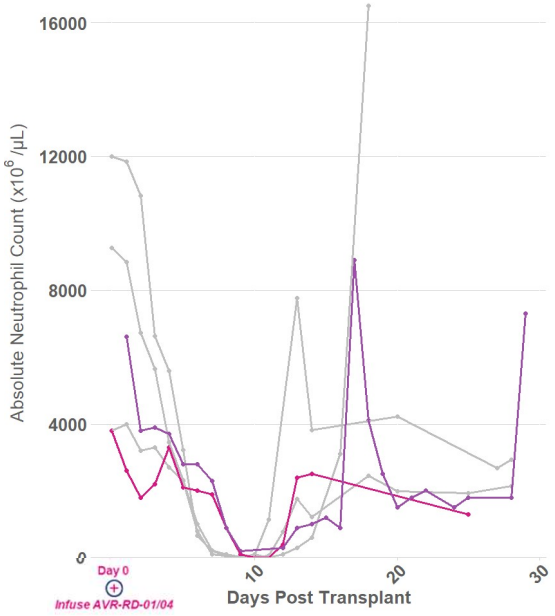
plato® UPGRADE 2

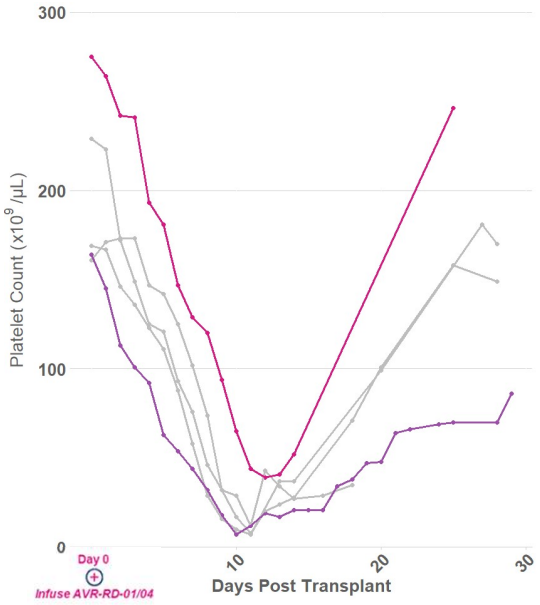
PRECISION CONDITIONING UPGRADE:

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

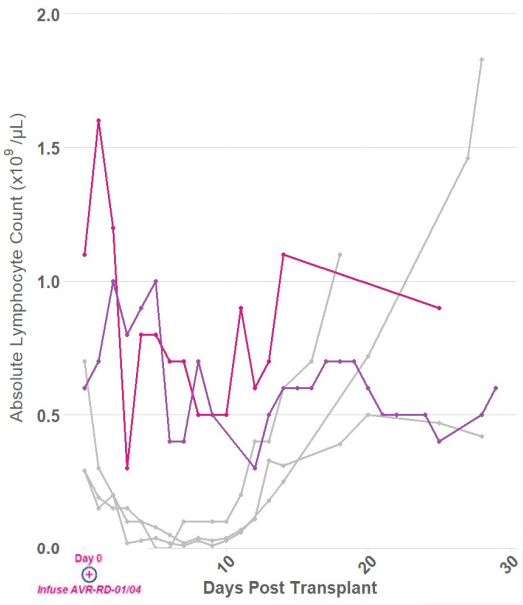
Absolute Neutrophil Count (ANC)



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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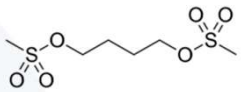
plato®
UPGRADE
2

PRECISION CONDITIONING UPGRADE:

Designed to access “hard-to-reach” compartments

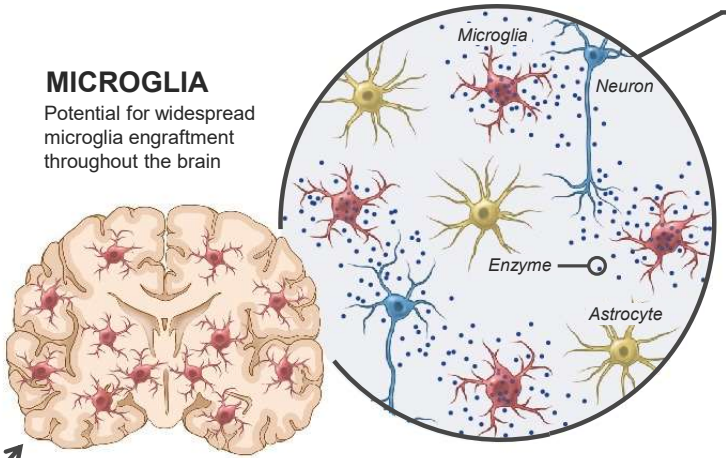
BRAIN

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



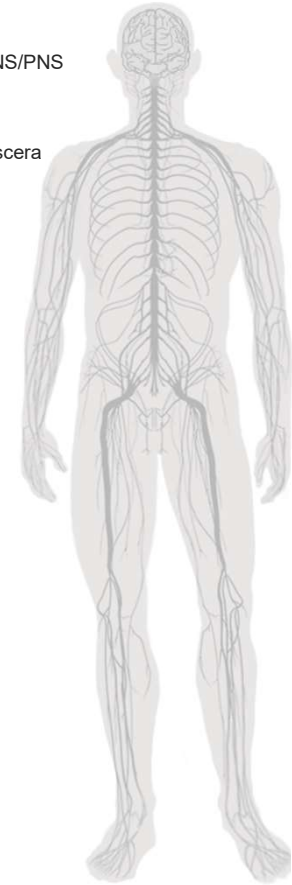
MICROGLIA

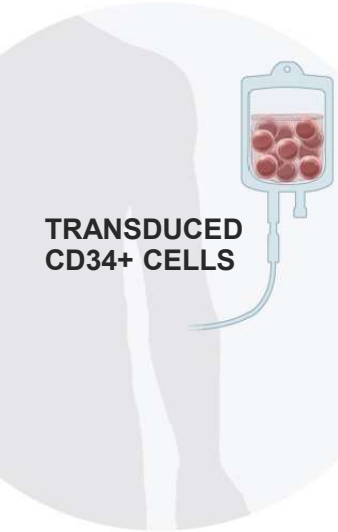
Potential for widespread microglia engraftment throughout the brain



CNS/PNS

Viscera



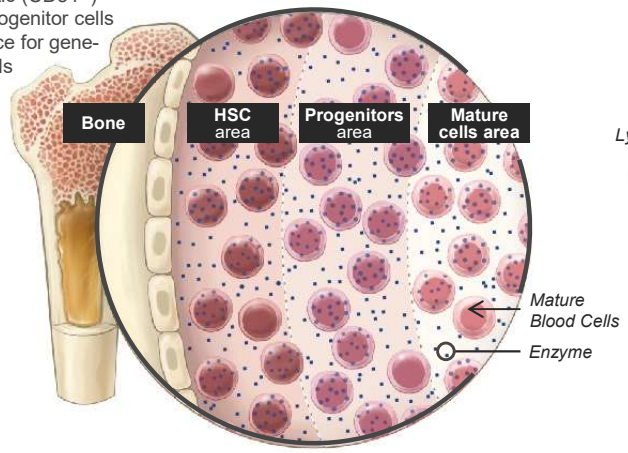


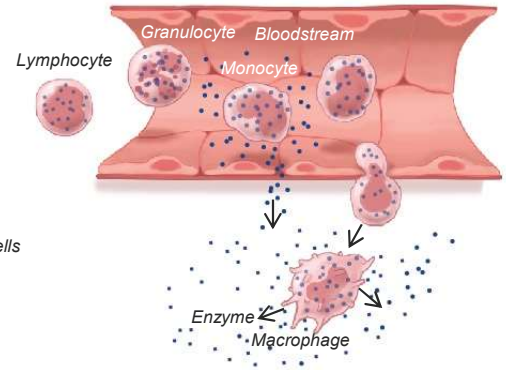
TRANSDUCED CD34+ CELLS

IN THE BONE MARROW

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

BONE MARROW → **PERIPHERAL TISSUE**





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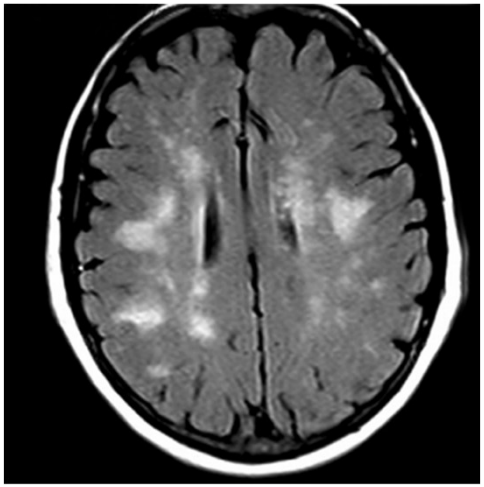
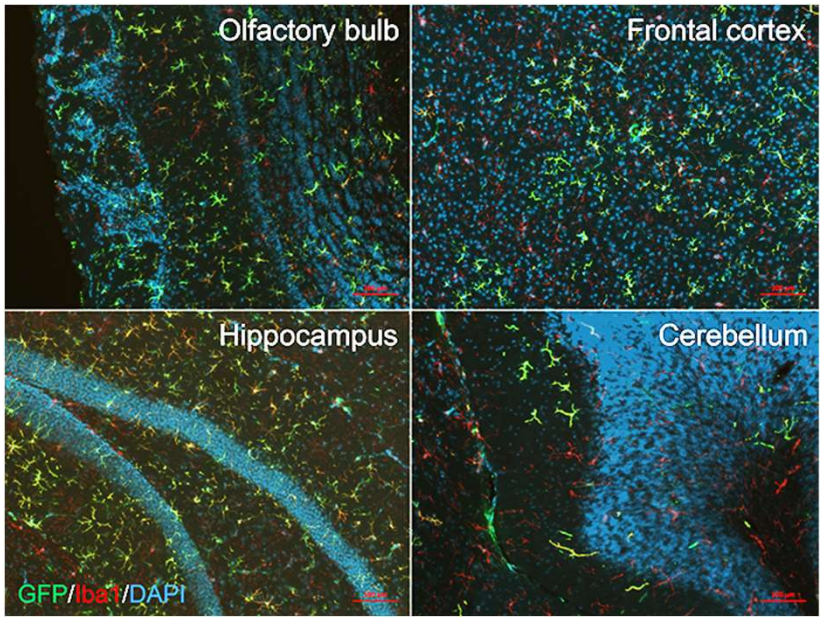
plato® UPGRADE 2

PRECISION CONDITIONING UPGRADE:

Designed to access “hard-to-reach” compartments, including the brain



MRI: 54 year old with Fabry disease demonstrating white matter lesions (WMLs)



GFP: Marker of engrafted cells
Iba1: Marker of microglia cells
DAPI: Nuclear stain irrespective of cell type

Global microglial coverage of mouse brain at 4 months post gene-modified HSC transplantation

- Widespread engraftment in regions critical for cognitive, motor, olfactory, and visual function
- Engrafted microglia/microglia-like cells have comparable morphology and classical lineage markers with endogenous microglia

Source: Buechner S, J. Neurol, Neurosurg, Psychiatry, 2008
MRI: Magnetic Resonance Imaging; ERT: Enzyme Replacement Therapy; WMLs: White Matter Lesions; HSC: Hematopoietic Stem Cell

plato®
UPGRADE
3

AUTOMATION UPGRADE:

Automated, scalable manufacturing system

Designed to elevate quality and overcome historic CMC bottlenecks

Expanded Scale

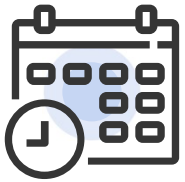
Potential to reach thousands of patients per year

Broader Reach

Portable platform designed for flexible global production using low grade clean rooms

High Quality

Automated, closed system designed to improve quality and consistency



Enhanced Convenience

Cryopreservation simplifies logistics and patient scheduling

Lower Costs

Designed to create efficiencies in vector design / scalable cell and vector production

POWERED BY AVROBIO plato

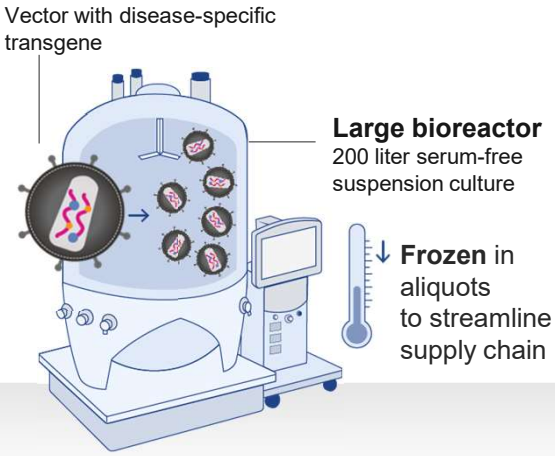
plato® UPGRADE 3

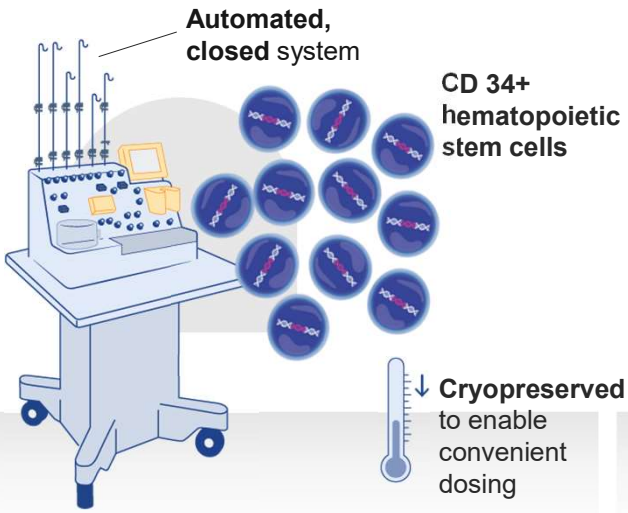
AUTOMATION UPGRADE:

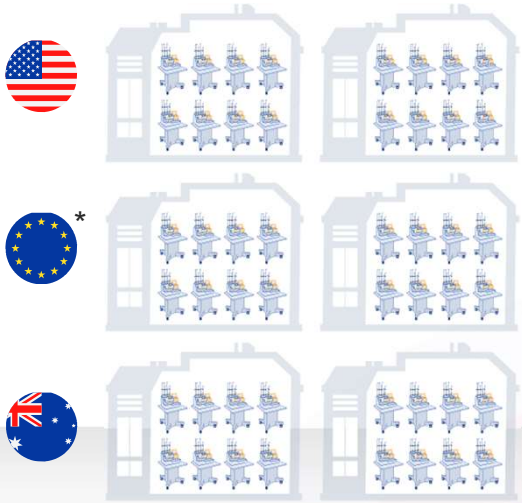
Designed to deliver large-scale manufacturing

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

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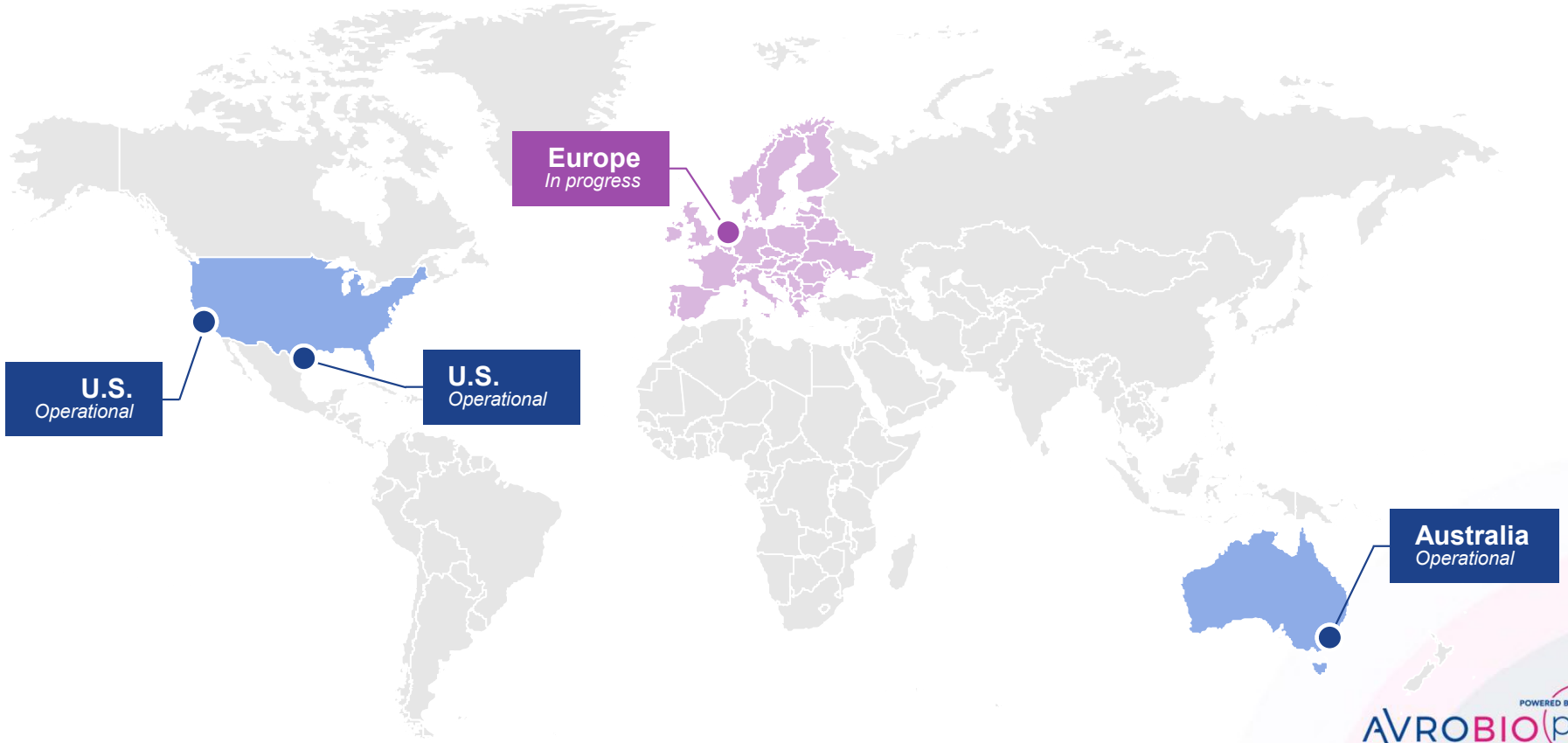
* European manufacturing capabilities planned for 2H 2020; manufacturing capabilities currently in place in U.S. & Australia

plato®
UPGRADE
3

AUTOMATION UPGRADE:

Global manufacturing established

Automated systems operational in 3 sites with 4th in progress



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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

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plato®
UPGRADE
1, 2, 3

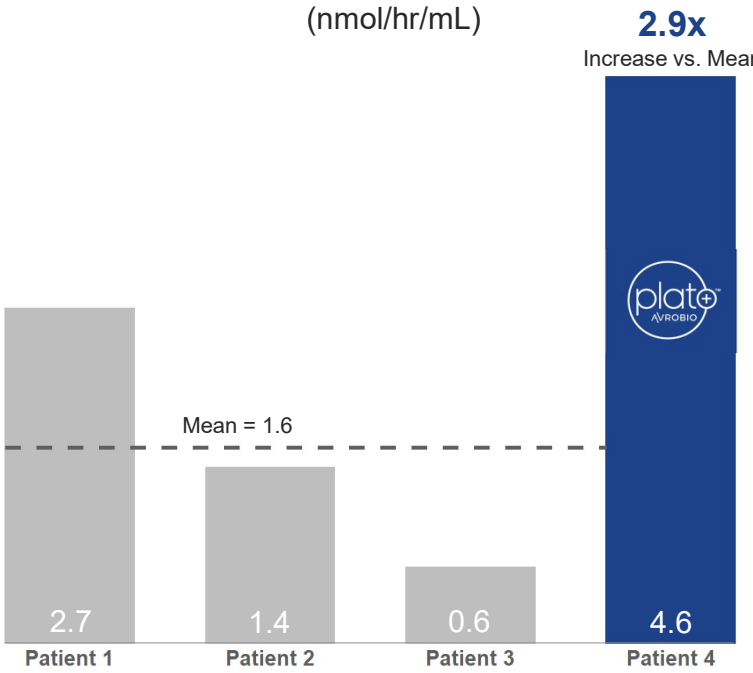
3 UPGRADES IN PLACE:

plato® metric compared to academic process

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

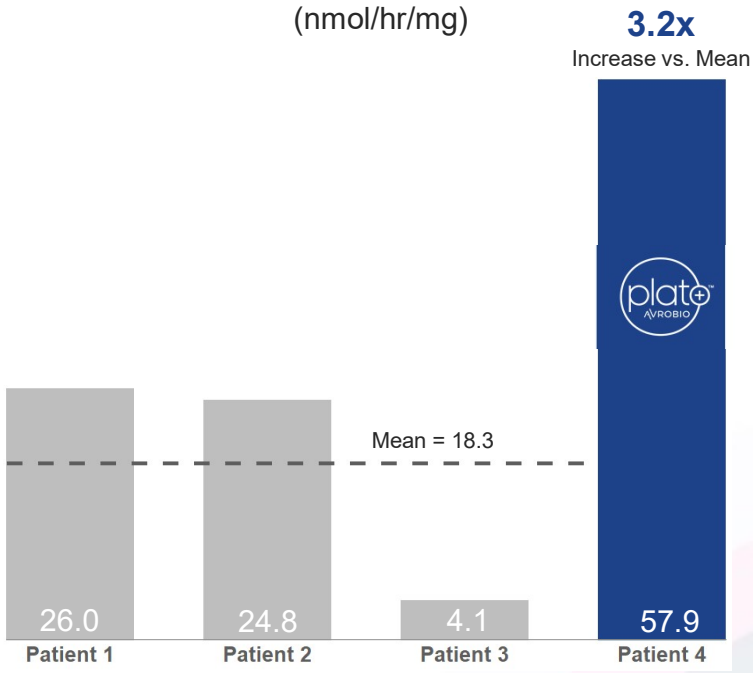
Plasma Enzyme Activity

(nmol/hr/mL)



Leukocyte Enzyme Activity

(nmol/hr/mg)



FAB-201: AVR-RD-01 Study

Milestones anticipated across the pipeline in 2020

Clinical activities and timelines subject in all respects to ongoing and evolving COVID-19 pandemic*

FABRY

- Continue recruitment in FAB-201 Phase 2 clinical trial
- Continue to report data across Phase 1 and Phase 2 clinical trials

GAUCHER

- Continue recruitment in GuardOne Phase 1/2 clinical trial
- Report initial patient data in H2 2020

CYSTINOSIS

- Continue recruitment in investigator-sponsored Phase 1/2 clinical trial
- Continue to report patient data

POMPE

- Complete preclinical IND-enabling activities

AVROBIO to hold first R&D Day in 2020

* For additional information, see the Company's Quarterly Report on Form 10-Q filed with the SEC on May 7, 2020.

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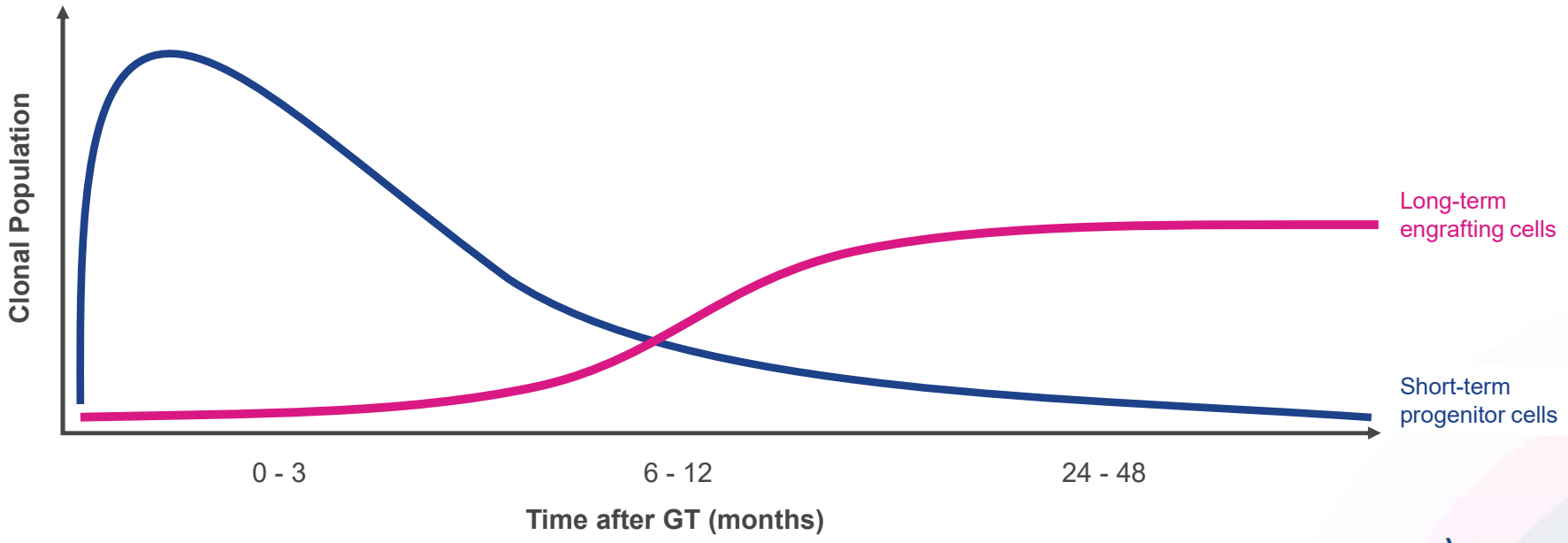
Appendix

Hematopoietic reconstitution occurs in two distinct phases

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

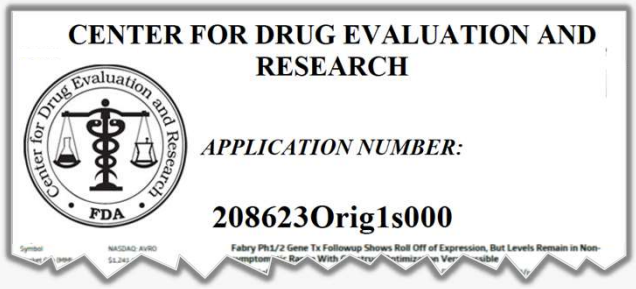
First wave of short-term progenitor cells start to exhaust with progressive takeover by a smaller population of long-term engrafting cells



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

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


CENTER FOR DRUG EVALUATION AND RESEARCH

APPLICATION NUMBER:

208623Orig1s000

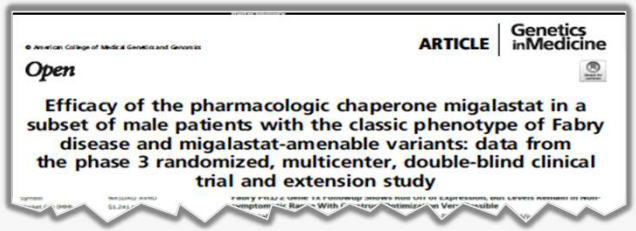
45 Amenable patients* (16 males / 29 females)

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)


ARTICLE | Genetics inMedicine

Open

Efficacy of the pharmacologic chaperone migalastat in a subset of male patients with the classic phenotype of Fabry disease and migalastat-amenable variants: data from the phase 3 randomized, multicenter, double-blind clinical trial and extension study

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

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[b] 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)

- *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*

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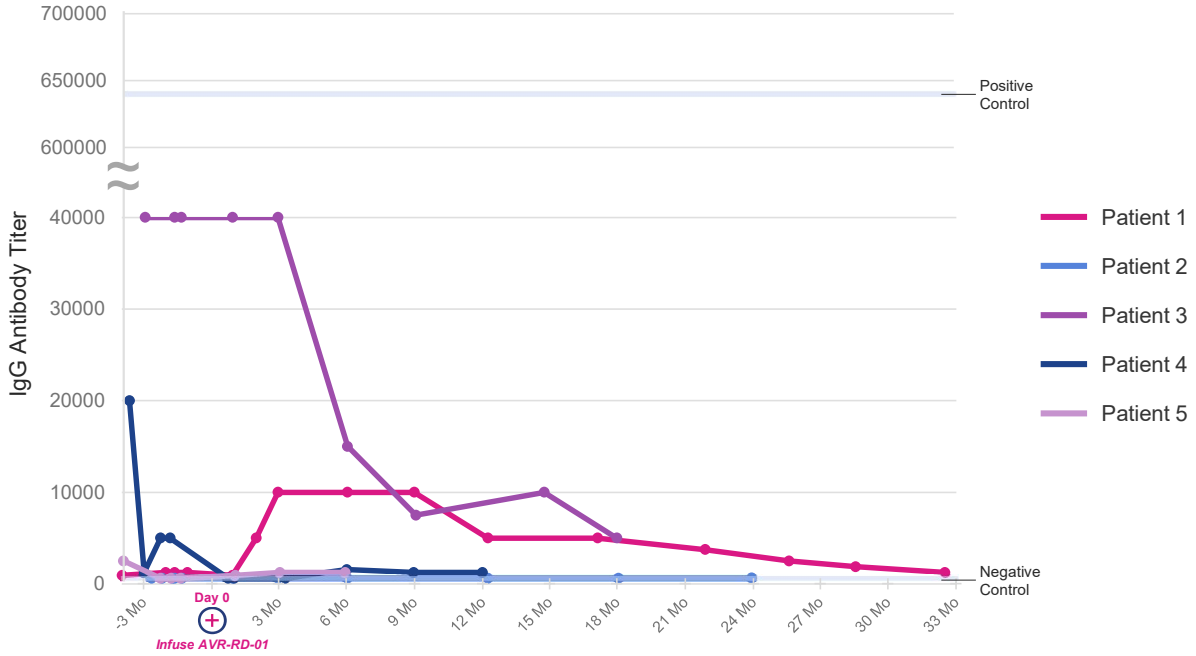
Reduction of pre-existing anti-ERT drug IgG antibodies following AVR-RD-01

Suggests potential as a therapeutic option independent of pre-existing antibodies

Fabry Disease Phase 1 IgG Antibody Titer



Similar Results Observed in Other Studies

San Raffaele Telethon Institute for Gene Therapy (SR-TIGET)

Change in pre-existing antibodies reported for Hurler disease (MPS-1)

- Ex vivo LV-CD34+ gene therapy with conditioning
- N = 6
- Evaluable patients (5/6) demonstrated sustained, supraphysiologic blood IDUA activity
- 4/5 prior ERT (rhIDUA) exposure (5-28 months)
- 4/5 pre-existing ERT-induced IgG antibodies
- 6/6 anti-rhIDUA IgGs undetectable 2 months post gene therapy

Source: *Gentner B et al., Blood, 2019*

ERT: Enzyme Replacement Therapy; IgG: Immunoglobulin G; MPS-1: Mucopolysaccharidosis Type 1; IDUA: Iduronidase: SR-TIGET: San Raffaele Telethon Institute for Gene Therapy; LV: Lentiviral; rhIDUA: Recombinant Human alpha-L-Iduronidase

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