AVROBIO

Freedom from a lifetime of disease

February 6, 2019

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Today's agenda

- Expanded leadership team
- Updated Fabry clinical data
- Introduce **plato**™
- Pipeline update

Steady stream of **clinical programs**

Worldwide rights across portfolio

Program	Proof-of-Concept	IND-Enabling	Phase 1/2	Pivotal	Expected Next Milestone
Fabry AVR-RD-01	Phase 1 Phase 2				Additional Ph 1 and 2 patient data
Gaucher AVR-RD-02					Initiate Ph 1/2 clinical trial
Cystinosis AVR-RD-04					Initiate Ph 1/2 clinical trial
Pompe AVR-RD-03					Advance preclinical program

Cell, gene and rare disease **industry leaders**

AVROBIO expands and strengthens team

MANAGEMENT TEAM

Geoff MacKay
President and CEO

Organogenesis inc. NOVARTIS

Birgitte Volck, PhD, MD
President of Research and Development

gsk sobi AMGEN

Kim Warren, PhD
Head of Operations

Lonza ccrm

Erik Ostrowski
Chief Financial Officer

Summit therapeutics LEERINK Organogenesis inc.

Chris Mason, MD, PhD, FRCS
Chief Science Officer

UCL

Steven Avruch, JD
General Counsel

Biogen

Deanna Petersen, MBA
Chief Business Officer

Shire agenus

Josie Yang, PhD
Head of Regulatory Affairs

PTC THERAPEUTICS FDA ImClone Systems

Kathryn McNaughton, PhD
SVP Portfolio & Program Management

Shire Synageva

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Patient focus groups December 2018

Significant unmet need and continued disease progression on ERT

"I feel awful and there [are] so many, so many things that don't show up on tests."

"I have severe pain, severe exhaustion."

"My reason for doing ERT is not so like I'll feel better. I wish it was. It would be great if it lessened my pain or something. It is longevity of life, period."

"The GI issues and pain, the infusions don't really help with that."

"[ERT] is not going to help a stroke."

"My mother has Parkinson's. My brother is 34 and 2 weeks ago because of his Gaucher's, he just had a double hip replacement."

"I have avascular necrosis in both my hips."

"A day or two before I have my treatment, I don't notice as much. My partner notices. You look white as a ghost. I'm exhausted. I'm snappy and I clearly had 8 hours of sleep, but no part of me feels that way."

Patients with **Fabry disease**

Patients with **Gaucher disease**

Source: *Health Care Utilization and Satisfaction with Treatment, AVROBIO Focus Group, Dallas, TX, December, 11 2018.*

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Our approach

One platform applied across our portfolio

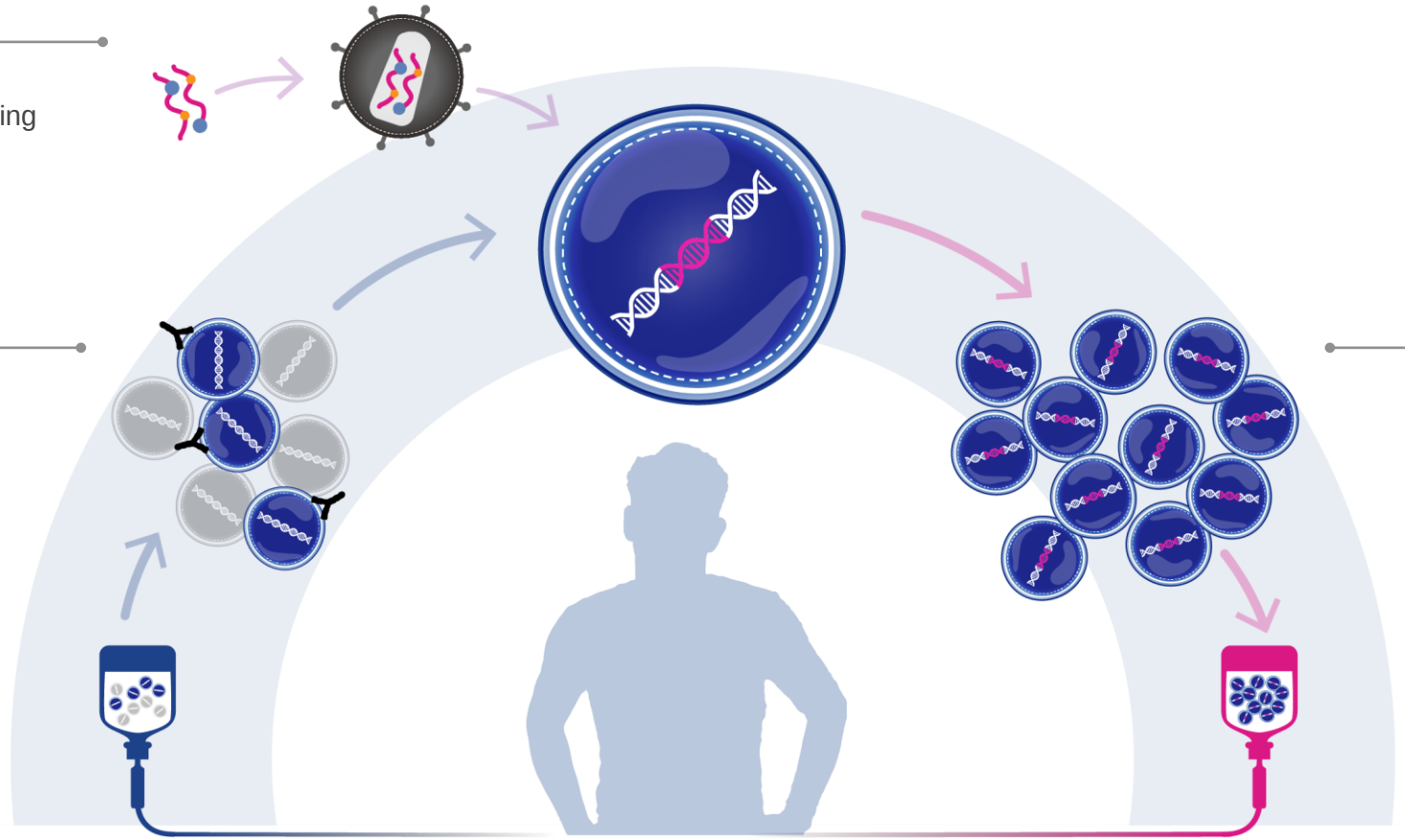
3 **Transduce** with lentiviral vector carrying normal gene

2 **Select** CD34+ stem cells

1 **Collect** mobilized blood

Harvest and freeze **4**

Infuse **5**



GENE THERAPY PLATFORM

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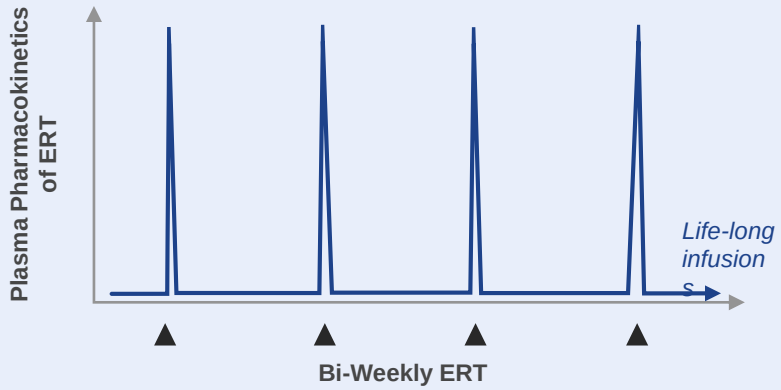
Life-long treatments vs. Potential **single dose cure**

	DISEASE PROGRESSION **CONTINUES**	DISEASE PROGRESSION **COULD HALT**
	Enzyme Replacement Therapy (ERT) Temporary bolus of enzyme, not curative	**AVROBIO Gene Therapy** 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

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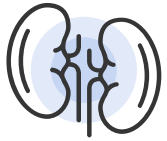
AVRO-RD-01 in Fabry disease

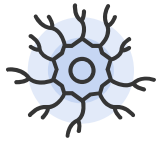
Goals for gene therapy in **Fabry disease**

TO PREVENT OR IMPROVE:



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

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

CNS complications
Unmet needs: TIA/stroke, depression, mild cognitive deficiency, white matter hyperintensities

Sources: *Wanner C et al, Med Genetics and Metab, 2018; Burlina A, JIEMS, 2016*

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New clinical data

Growing body of clinical data in Fabry

- All patient data to-date demonstrated **AGA enzyme activity** above the diagnostic range
- **Substantial reductions in substrate and metabolite levels** of patients on gene therapy alone observed across multiple measurements
- Impact seen in **both** treatment-naïve patients and previously treated patients who have discontinued ERT
- AVR-RD-01 observed to be **generally well tolerated**

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AVR-RD-01 **Fabry clinical trials**

6 patients dosed across Phases 1 and 2

PHASE 1 Investigator-Sponsored Trial*	**PHASE 2** AVRO – FAB-201 Trial
Patients **n = up to 6** **On ERT > 6 months prior to enrollment** **18-50 year-old males**	**Patients** **n = 8-12** **ERT-naive** **≥ 16 year-old males**
Key Objective Safety	**Key Objectives** Safety and efficacy

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** Sponsored by FACTS team (Fabry disease Clinical research and Therapeutics) in Canada*

Phase 1: Investigator-sponsored study* in **ERT-treated Fabry patients**

4 patients dosed to-date

ERT-TREATED FABRY PATIENTS

Day -30
Stop ERT
Mobilize stem cells

Day -1
Conditioning

Day 0
Infuse AVR-RD-01

Day 28
Post-treatment assessment

Day 30
Re-start ERT

Months
1, 3, 6, 12, 18, 24, 36, 48 & 60
Safety & efficacy assessments

Clinical pilot study of autologous stem cell transplantation of CD34+ cells engineered to express AGA in patients with Fabry disease

OBJECTIVES	PATIENTS	ASSESS
• Safety • Preliminary efficacy	• Up to 6 patients • 18-50 year old males • Receiving ERT	• Plasma and leukocyte enzyme activity • Presence of vector in peripheral blood and bone marrow cells • Safety

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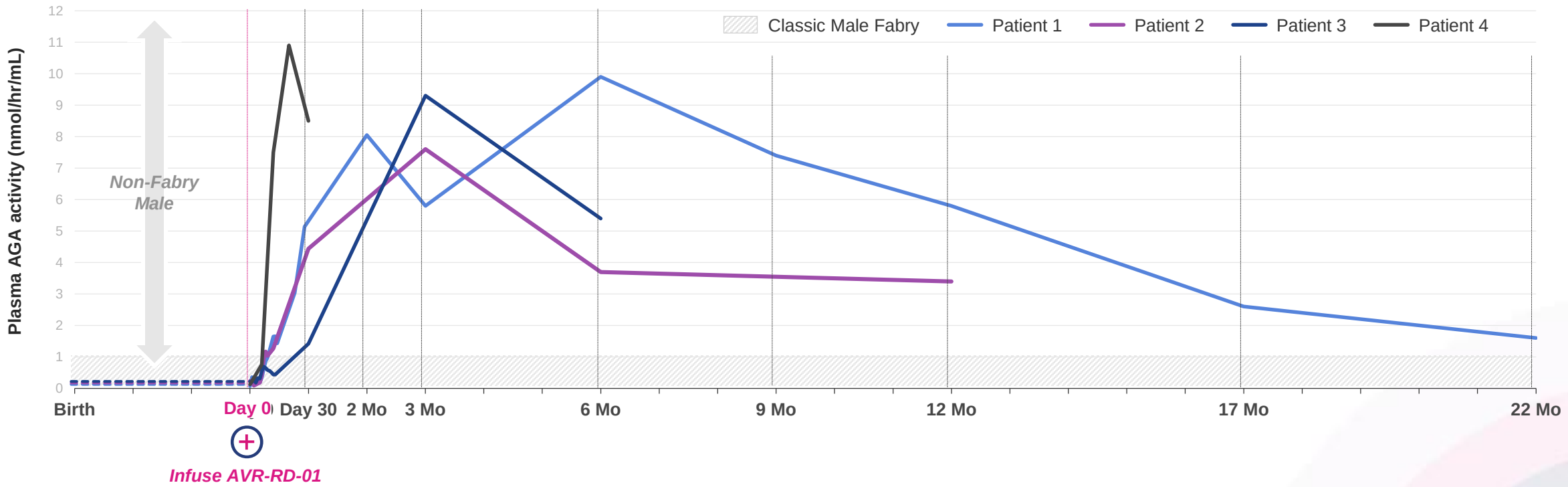
** **Note:** Protocol amendment allows for discontinuation of ERT 6 months after treatment with AVR-RD-01*
** Sponsored by FACTs team (Fabry disease Clinical research and Therapeutics) in Canada*

Phase 1: Substantial **enzyme activity elevation**

Sustained at 22 Months

Level of AGA enzyme activity rose from nearly undetectable levels to levels above the range for males with classic Fabry disease



Source of reference bar: *Tsukimura T et al, Mol Genet Metab, 2014*
Note: *Enzyme measurements are taken at ERT troughs*
Note: *Dotted line illustrative only*

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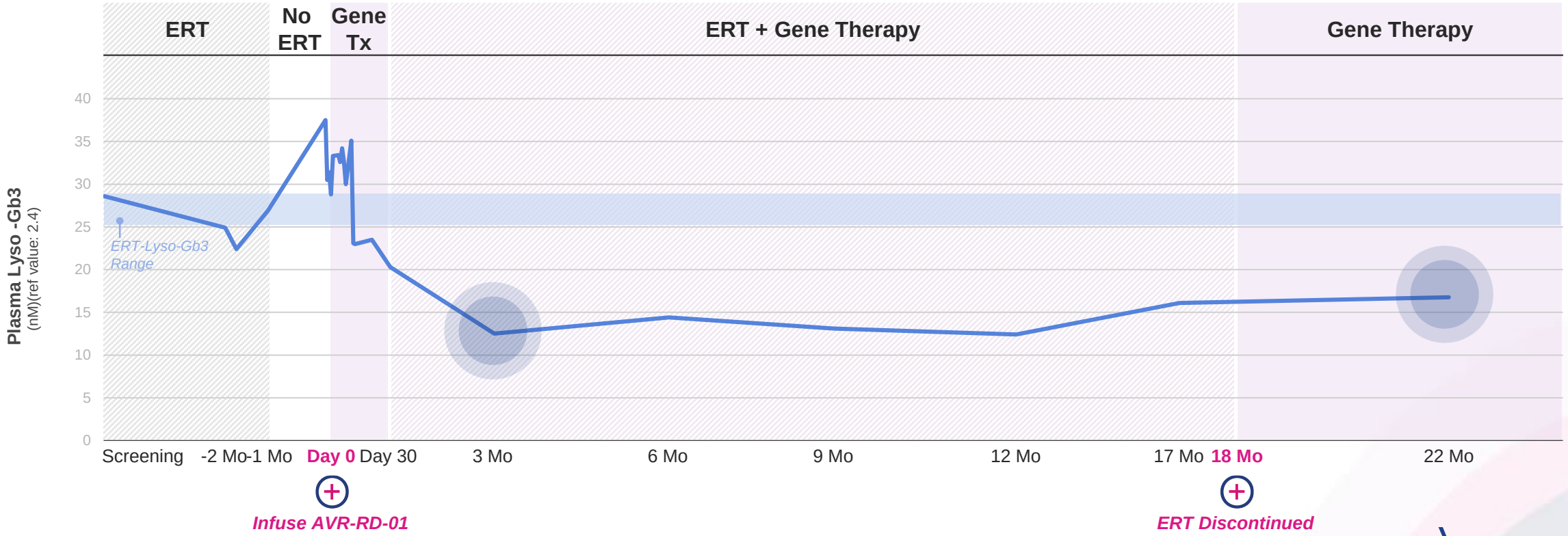
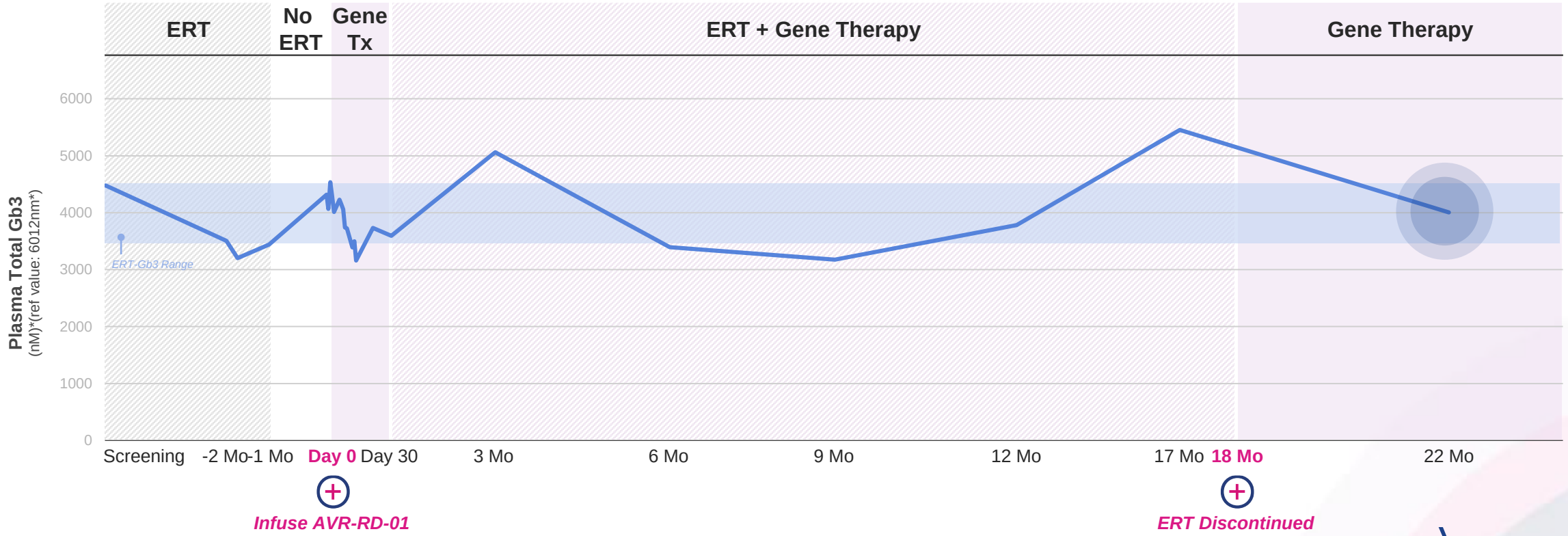
Phase 1: Plasma Lyso-Gb3 **reduction sustained** after discontinuation of ERT

PATIENT #1: *Plasma Lyso-Gb3 remains below ERT levels with AVR-RD-01* ***gene therapy alone***



Phase 1: Plasma Total Gb3 Levels **sustained** after discontinuation of ERT

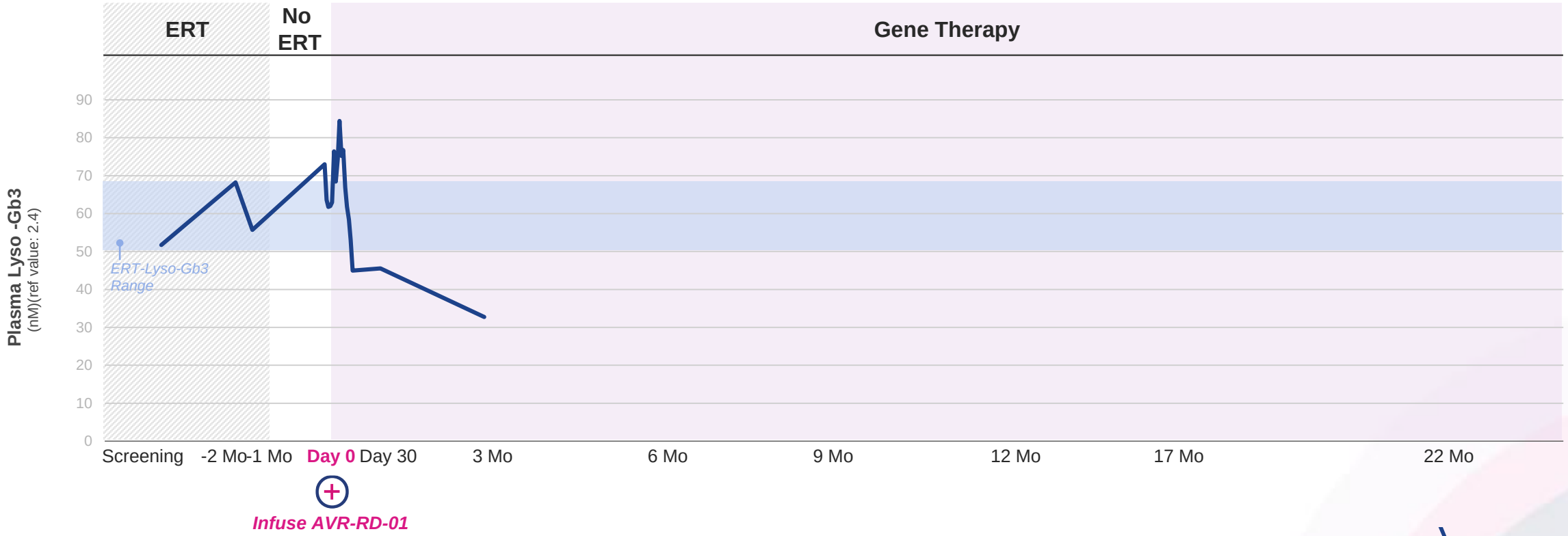
PATIENT #1:** Plasma Total Gb3 remains at ERT levels with AVR-RD-01 **gene therapy alone



* Ref value updated Aug. 2018 to 4961

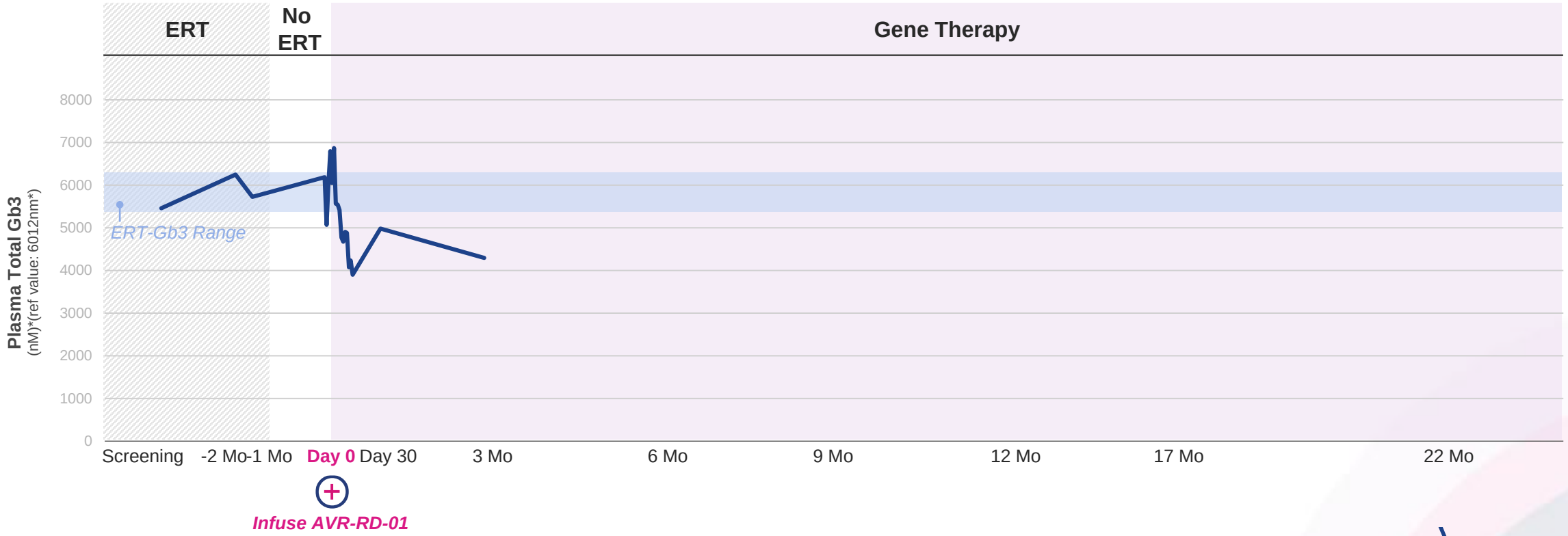
Phase 1: Plasma Lyso-Gb3 declines below levels on ERT with **gene therapy alone**

PATIENT #3: *Did* ***not resume ERT*** *treatment following AVR-RD-01 dosing*



Phase 1: Plasma Total Gb3 declines below levels on ERT with **gene therapy alone**

***PATIENT #3:** Did **not resume ERT** treatment following AVR-RD-01 dosing*



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** Ref value updated Aug. 2018 to 4961*

Phase 1: Vector Copy Number (VCN)

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

Peripheral Blood VCN	Patient 1	Patient 2	Patient 3
1 Month	0.4	0.8	0.2
3 Months	0.6	1.1	0.8
6 Months	0.4	0.4	0.5
9 Months	0.3	-	
12 Months	0.2	0.4	
17 Months	0.1		
22 Months	0.1		

PATIENT #1: At 14 months, 13% of bone marrow mononuclear cells were vector positive

Phase 1: AVR-RD-01 **generally well tolerated**

Safety and tolerability

- **AEs reported as expected** for melphalan conditioning
- **No SAEs related to AVR-RD-01**
- **No antibody elevation,** consistent throughout study for all patients

Note: *Anti-AGA antibodies were measured by ELISA to determine titers in plasma*

FAB-201: AVROBIO Phase 2 study in **Treatment-Naïve Fabry patients**

2 patients dosed to-date

TREATMENT-NAÏVE FABRY PATIENTS

Day -60
Mobilize stem cells

Day -1
Conditioning

Day 0
Infuse AVR-RD-01

Day 28
Post-treatment assessment

Months 2, 3, 6, 9 & 12
Safety & efficacy assessments

Phase 2 open-label, multinational study of the efficacy and safety of ex vivo lentiviral-based vector gene therapy AVR-RD-01 for treatment-naïve subjects with classic Fabry disease

OBJECTIVES	PATIENTS	ASSESS
• Safety • Efficacy (functional endpoints and biomarkers)	• 8-12 patients • Adult males (age ≥ 16 years) • Treatment-naïve	• Primary efficacy endpoint: reduction of substrate in kidney biopsy • Substrate reduction (Gb3 and/or lyso-Gb3) in urine, plasma, skin • Enzyme (AGA) activity • Kidney function • Cardiac size • Vector Copy Number (VCN) • Chimerism • Safety

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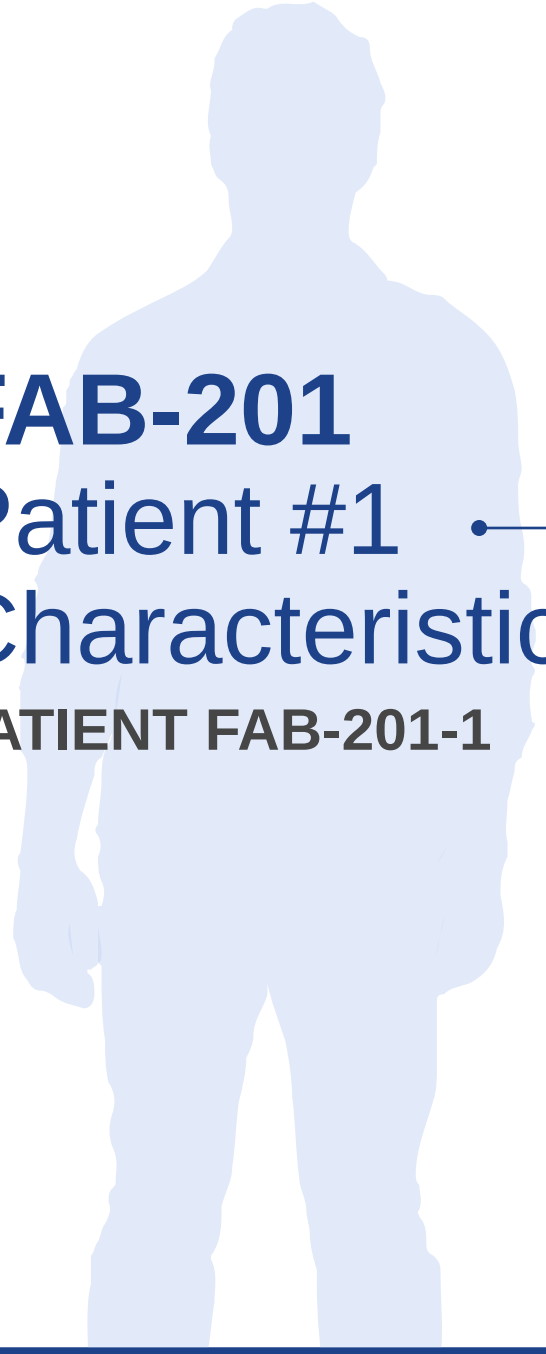

FAB-201 Patient #1 Characteristics

PATIENT FAB-201-1

GENERAL

- 21 year old male
- No prior treatment with ERT or chaperone therapy
- Family history of Fabry disease

FABRY DISEASE HISTORY

- Chronic acral pain and knee pain, onset age 10
- Gastrointestinal symptoms (intermittent diarrhea), onset age 15
- Diagnosed age 19
  - Umbilical keratoma
  - Decreased cold sensation

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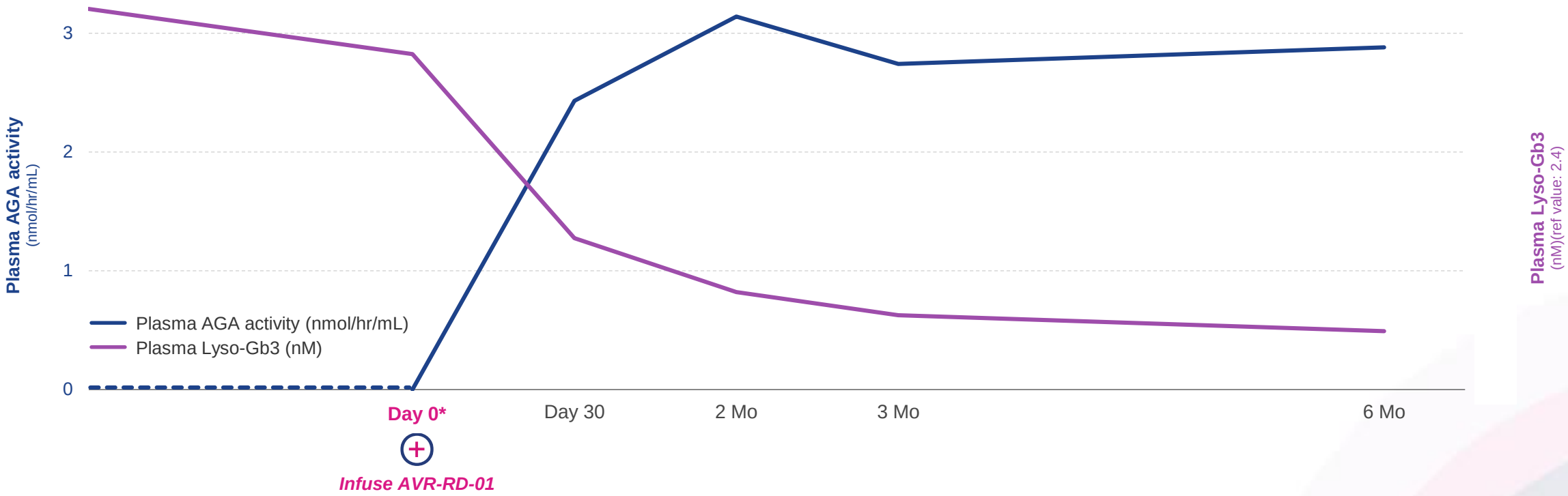
FAB-201: Substantial increase in AGA enzyme activity with associated **reduction in Plasma Lyso-Gb3**

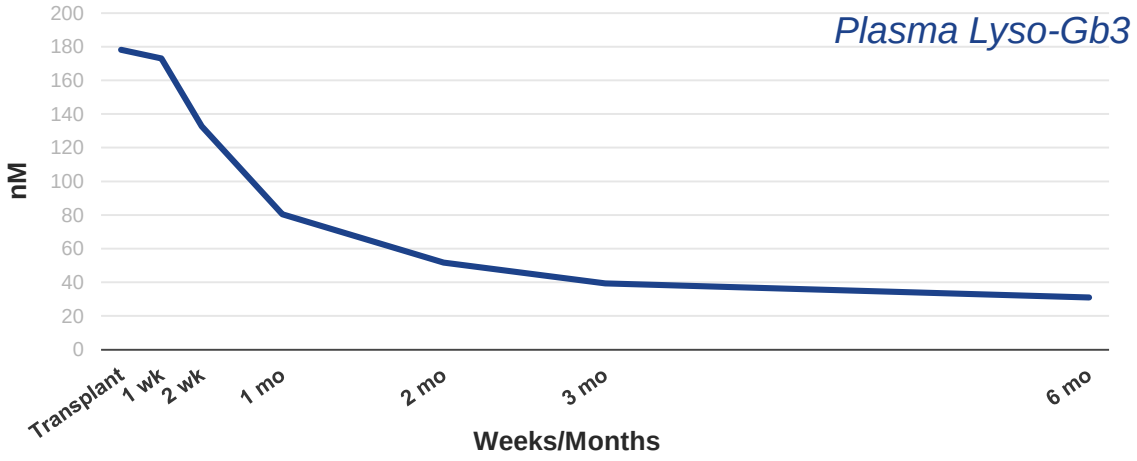
Patient #1: 85% reduction in plasma lyso-Gb3 levels observed within 6 months

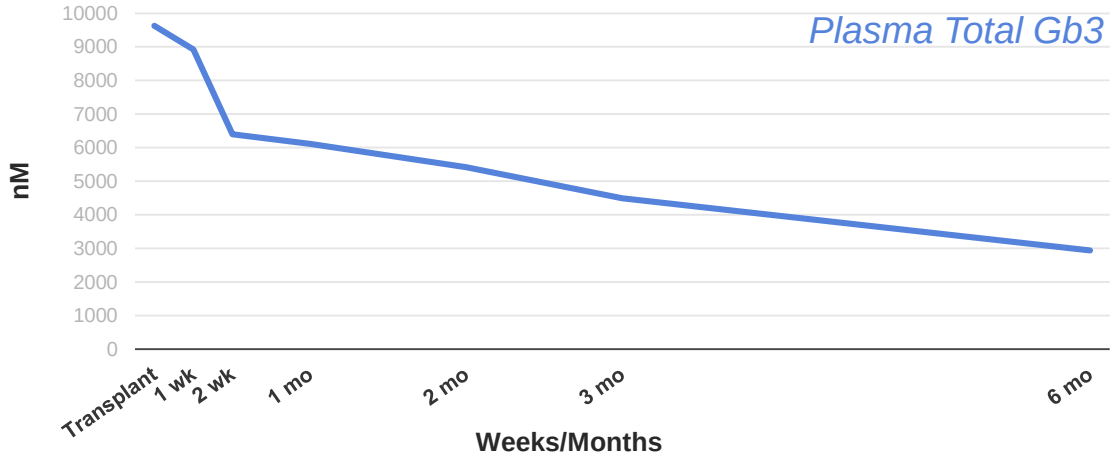


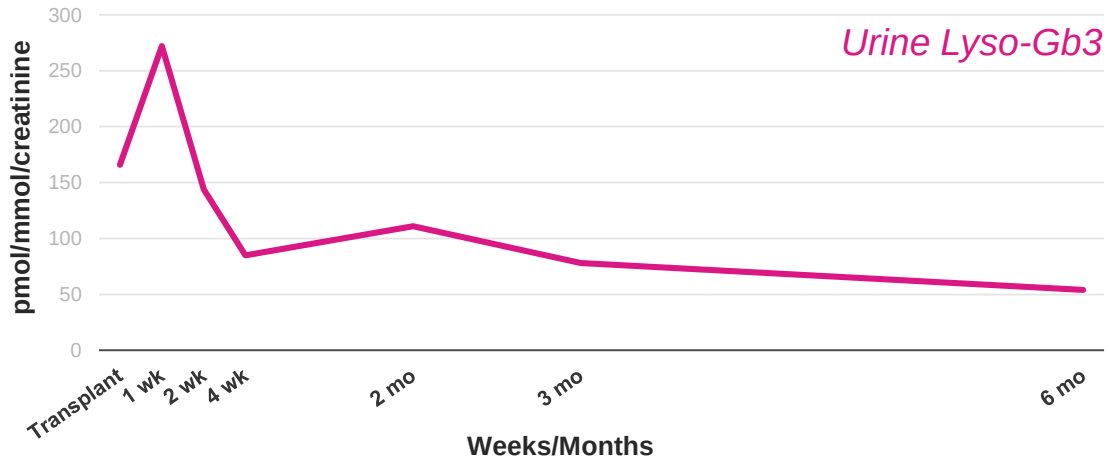
* Dotted line represents AGA activity measured in serum

FAB-201: Patient #1 – Decline in multiple substrate/metabolite levels following gene therapy

Gb3 and lyso-Gb3







FAB-201: Vector Copy Number (VCN)

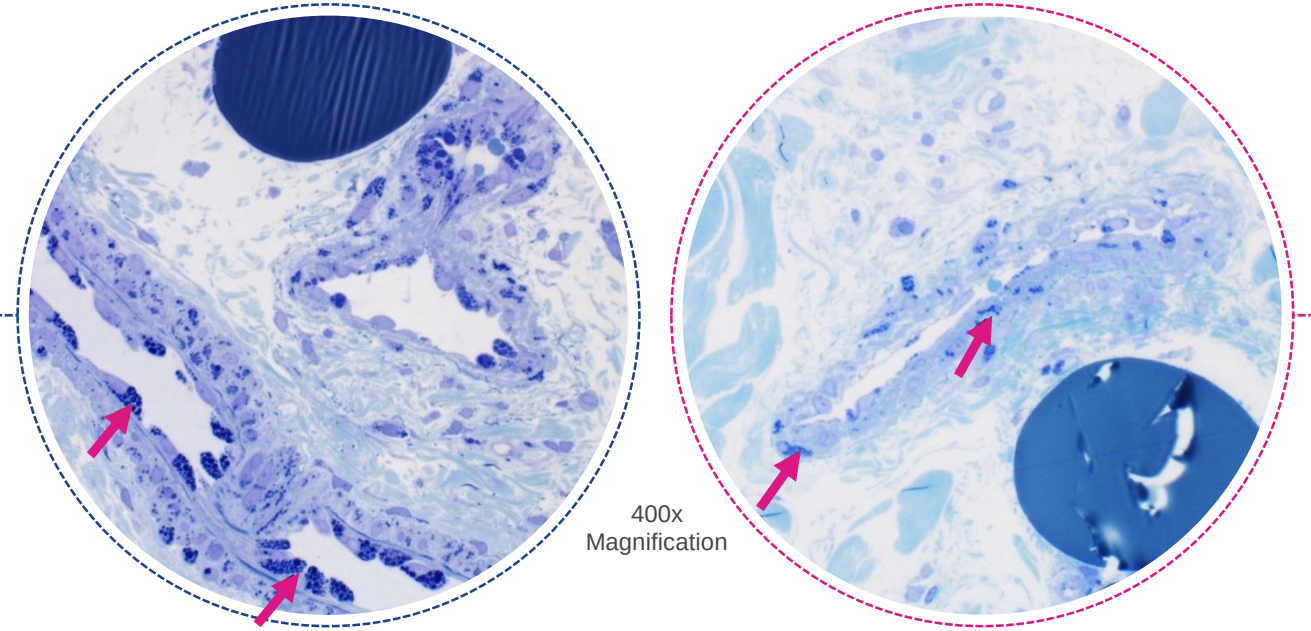
Drug Product VCN	
Patient FAB-201-1	0.7

Peripheral Blood Average VCN	Patient FAB-201-1
1 Month	0.2
2 Months	0.2
3 Months	0.5
6 Months	0.2

FAB-201: Reduction in substrate inclusions in skin endothelial cells

Patient #1 achieved reduction in skin biopsy score from 3 to 2 within 6 months

Baseline
Biopsy Score: 3
Severe accumulation



400x
Magnification

6-Month
Biopsy Score: 2
Moderate accumulation

Skin Biopsy Scoring:
3 = Large accumulations of inclusions, with some clusters at the juxtanuclear region and around cytoplasmic borders, and bulging of the vessel lumens
2 = Multiple vessels with multiple sites of single or multiple inclusions
1 = Majority of vessels with a single endothelial inclusion
0 = None or only trace microvascular endothelial deposits of GL-3 (normal or nearly normal)
***Source**: Thurberg B et al, J Investigative Dermatology, 2004*

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Phase 2: AVR-RD-01 **generally well tolerated**

Safety and tolerability

AEs reported as expected for melphalan conditioning

2 serious adverse events reported

- one pre-treatment and one post-treatment (dehydration, nausea and vomiting) potentially related to conditioning, but not considered to be related to AVR-RD-01

No antibody elevation

- Baseline and 4-week values negative

Note: *Anti-AGA antibodies were measured by ELISA to determine titers in plasma*

FAB-201 SUMMARY

PATIENT FAB-201-1 at 6 months

FAB-201: Phase 2 in treatment-naïve Fabry patients

- Substantial enzyme increase
- Associated with substrate reduction
- Generally well tolerated
- Awaiting long-term follow-up

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Significant advances in Fabry clinical program

Growing body of clinical data in Fabry

- **6 patients** dosed across **2 active clinical trials**
- All patient data to-date demonstrated **AGA enzyme activity** above the diagnostic range
- **Substantial reduction in substrate and metabolites** observed in both ERT-treated and ERT-naïve patients
- AVR-RD-01 observed to be **generally well tolerated**

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INTRODUCING

plato™

Gene therapy.
Evolved.

AVROBIO

Introducing **plato**™

AVROBIO's foundation for worldwide commercialization

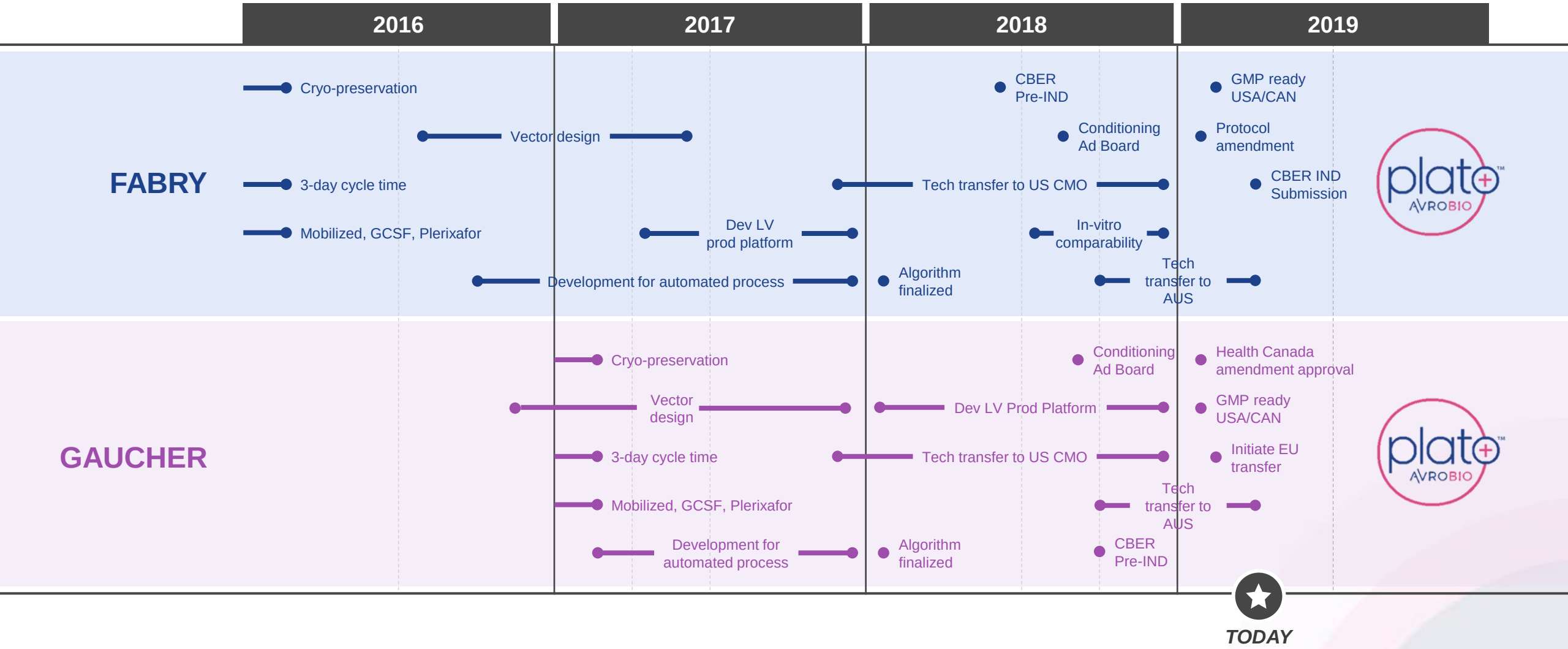
- A **vector system** and cell **manufacturing solution** designed to support commercialization
- **Automated, closed manufacturing system** for CD34+ gene therapy
- Designed to safely deliver **long-term efficacy** and **durability**

plato™

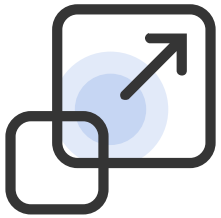
Gene therapy.
Evolved.

plato™: **Commercial** platform over **3 years** in the making

Clinical trials 2H 2019, regulatory milestone obtained

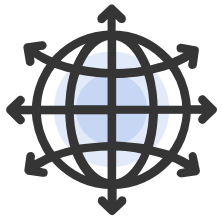


plato™ overcomes historical bottlenecks to enable commercialization



Expanded Scale

Potential to reach thousands of patients per year

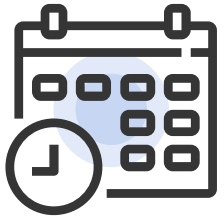


Broader Reach

Portable platform for flexible global production using low grade clean rooms

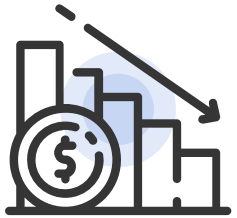
High Quality

Automated, closed system designed to improve quality and consistency



Longer Shelf-Life

Cryopreservation simplifies logistics and patient scheduling



Lower Costs

Efficiencies in vector design / scalable cell and vector production

plato™: Three 2019 upgrades designed to optimize **potency, safety, and durability**

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

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

** Average VCN per cell*
*** TDM (therapeutic drug monitoring)*

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plato™
Gene therapy.
Evolved.

UPGRADE 1

CONDITIONING UPGRADE:

plato™ transitions to busulfan TDM for anticipated advances in **safety and efficacy**

WHAT Switch from 100mg/m^2 melphalan to **busulfan** with **Therapeutic Drug Monitoring (TDM)**

WHY

- Busulfan **used successfully** in many gene therapy indications
- TDM intended to **elevate safety profile** while permitting higher intensity
- Potential to **impact CNS manifestations** which affect many LSD patients

SAFETY TRACK RECORD

Busulfan in **non-malignant** conditions

- Literature shows >700 patients with **NO reports** of t-MDS / t-AML
- **Isolated case** of t-MDS in a sickle cell patient in bluebird bio's gene therapy trial

t-MDS = Treatment-related myelodysplastic syndrome
T-AML = Treatment-related acute myeloid leukemia
References available upon request

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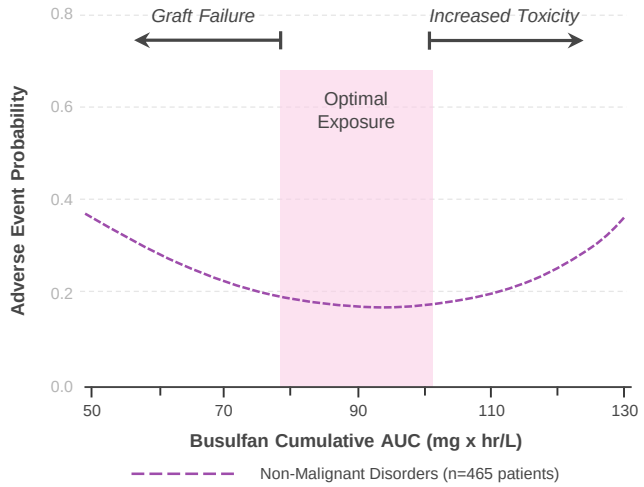
Gene therapy. Evolved.

UPGRADE 1

CONDITIONING UPGRADE:

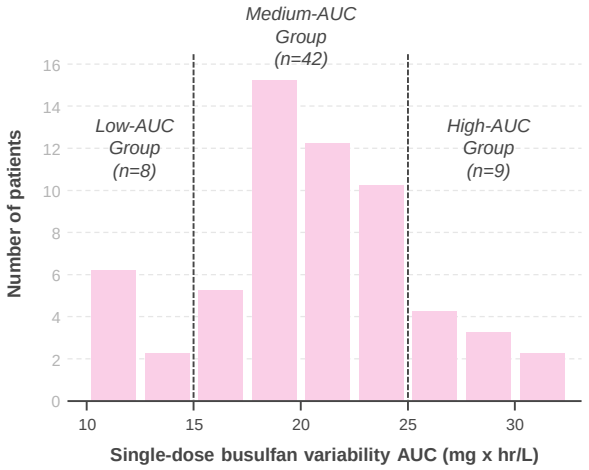
Busulfan intended to balance **engraftment** with **enhanced safety**

Optimized Dosing
Designed to Enhance Tolerability



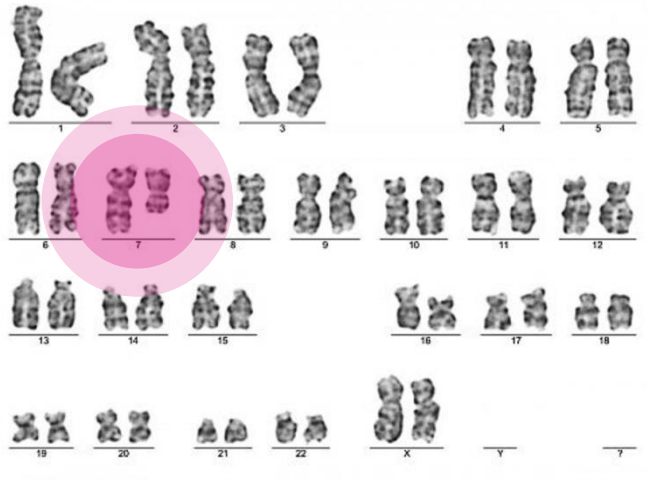
Lowest rate of complications in the Bu90 range

Optimized Monitoring
Designed to Enhance Safety



TDM via area under the curve (AUC) intended to eliminate out-of-bounds toxicity

Optimize Screening
Designed to Reduce Patient Risk



Myelodysplastic syndrome with excess blasts (MDS-EB-2)

Pre-screening for molecular and cytogenetic abnormalities has potential to further reduce risk

Sources: *Bartelink IH et al, Lancet Haematol, 2016; Kim B et al, Sci Rep, 2017; http://www.leukemia-cell.org/atlas*

AVROBIO

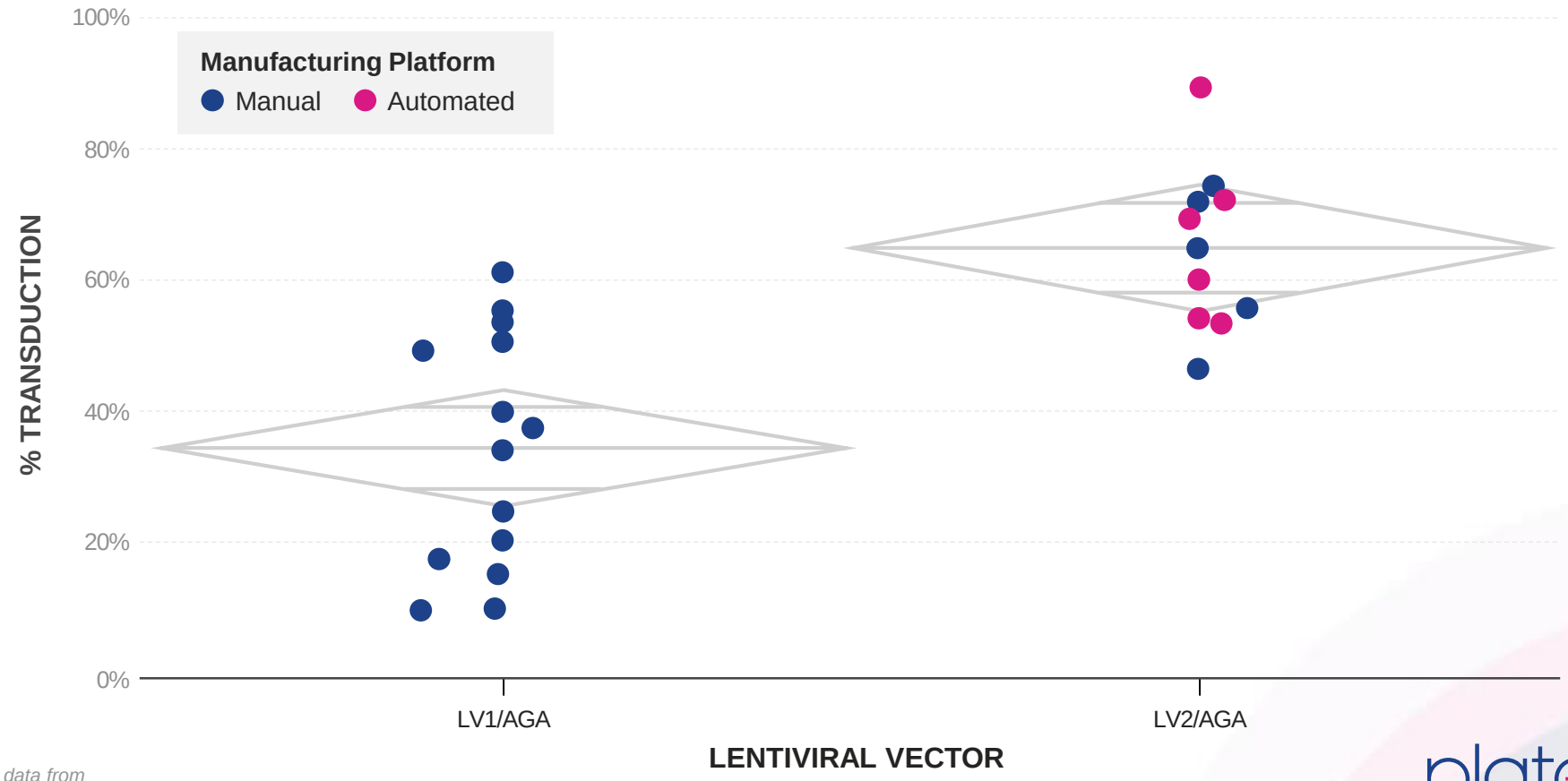
plato™
Gene therapy. Evolved.

UPGRADES 2 & 3

VECTOR & AUTOMATION UPGRADES:

plato™ designed to enhance **potency** and **long-term durability**

Increased
% Transduction



Note: *Data from all development runs using normal donor cells and data from four Fabry patients Drug Products are included, as of Oct 2018*

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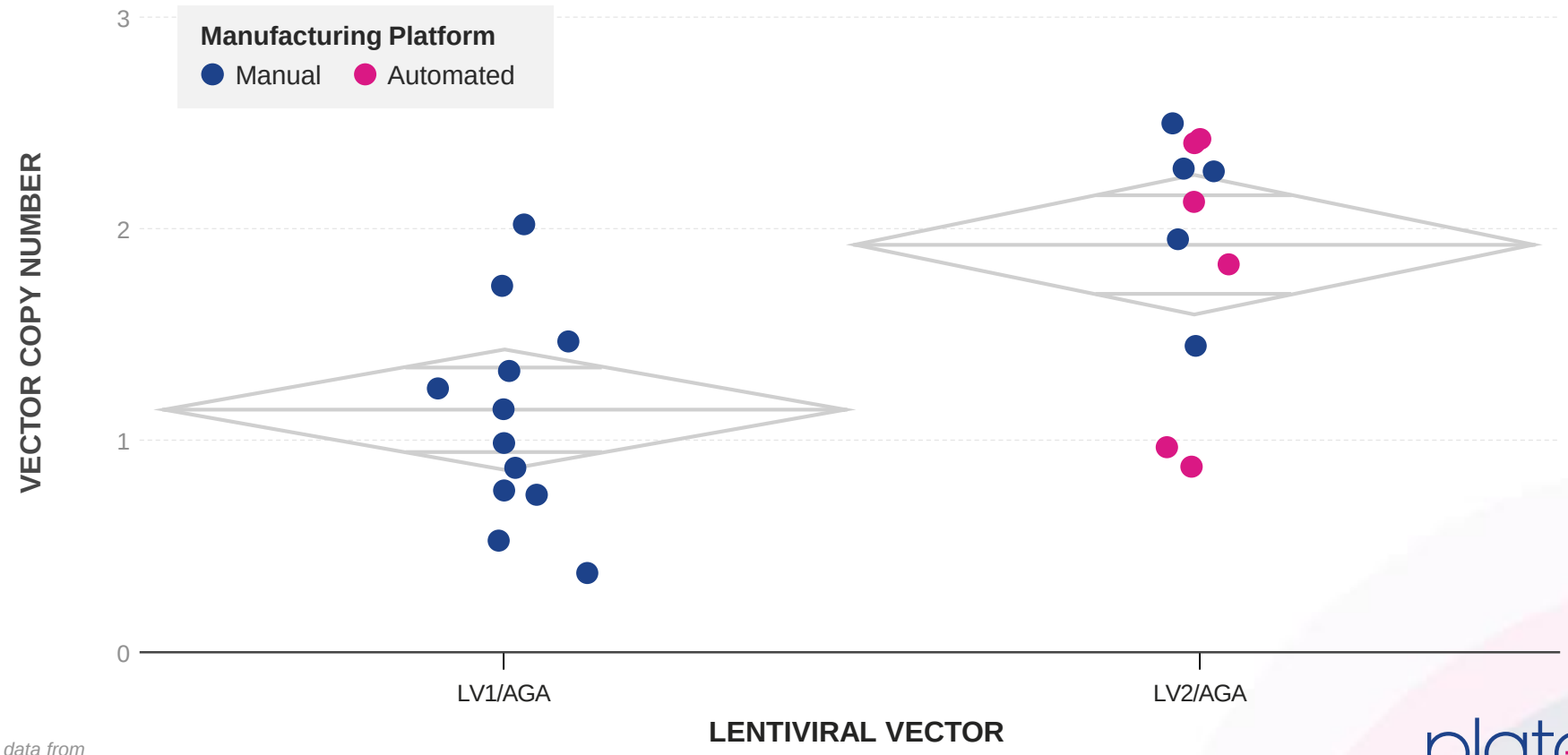
UPGRADES 2 & 3

VECTOR & AUTOMATION UPGRADES:

plato™ designed to enhance **potency** and **long-term durability**

Increased ***Vector Copy Number (VCN)***



Note: *Data from all development runs using normal donor cells and data from four Fabry patients Drug Products are included, as of Oct 2018*

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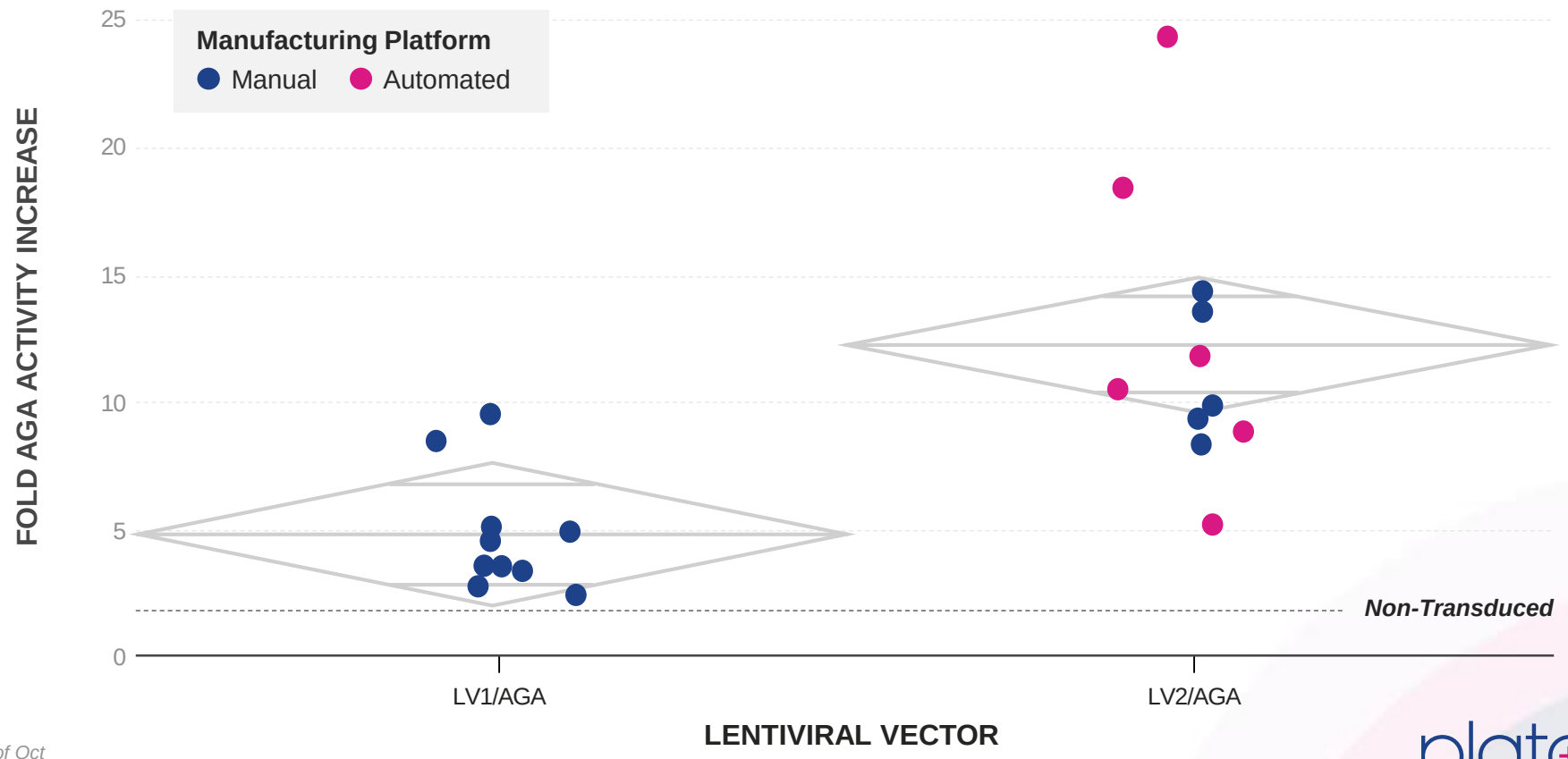
UPGRADES 2 & 3

VECTOR & AUTOMATION UPGRADES:

plato™ designed to enhance **potency** and **long-term durability**

Increased
AGA Enzyme Activity



Note: *Data from all development runs using normal donor cells, as of Oct 2018*

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Introducing **plato**™

AVROBIO's foundation for worldwide commercialization

- **Expanded Scale**
- **Broader Reach**
- **High Quality**
- **Longer Shelf-Life**
- **Lower Costs**

- A **vector system** and cell **manufacturing solution** designed to support commercialization
- **Automated, closed manufacturing system** for CD34+ gene therapy
- Designed to safely deliver **long-term efficacy** and **durability**

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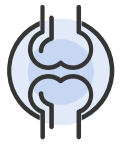
AVRO-RD-02 in Gaucher disease

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

TO PREVENT OR IMPROVE:



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

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

CNS complications
Unmet needs: GBA-Parkinson's disease

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*

AVROBIO

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

ERT-stable and treatment-naïve patients

GAUCHER DISEASE TYPE 1

plato™ AVROBIO

Day -60	Day -4	Day 0	Day 28	Months
Mobilize stem cells Patients on ERT: ERT discontinued D -14	Conditioning	Infuse AVR-RD-02	Post-treatment assessment	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 subjects with Type 1 Gaucher disease

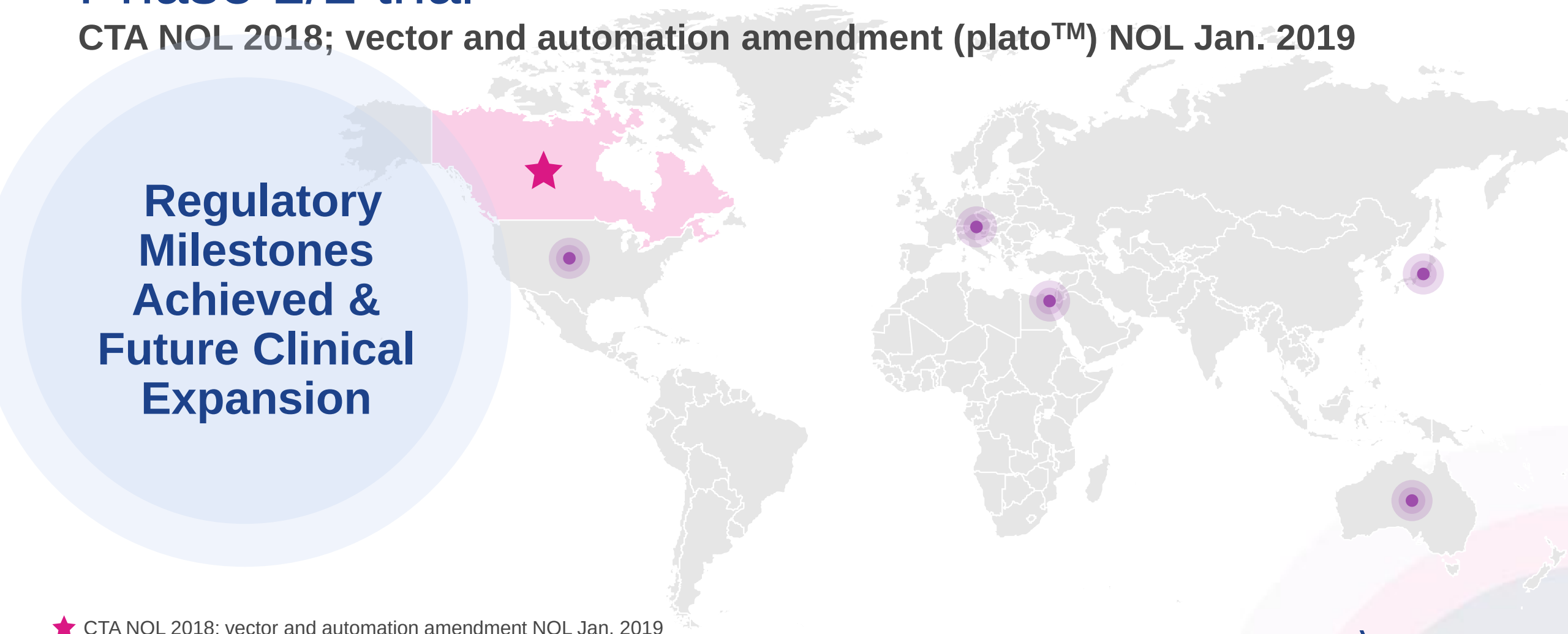
OBJECTIVES	PATIENTS	ASSESS
• Safety • Engraftment • Efficacy (functional endpoints and biomarkers)	• 8-16 patients • 16-35 year old males and females • Two arms – Treatment naïve – Stable receiving ERT	• Vector Copy Number (VCN) • Chimerism • GCase activity, including in CSF • Efficacy – Hematologic values – End-organ volumes and BMD – Biomarkers and QoL • Safety

AVROBIO

GAU-201: Regulatory milestones for Phase 1/2 trial

CTA NOL 2018; vector and automation amendment (plato™) NOL Jan. 2019

Regulatory Milestones Achieved & Future Clinical Expansion



★ CTA NOL 2018; vector and automation amendment NOL Jan. 2019

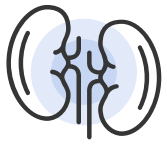
● Planned expansion

AVROBIO

AVRO-RD-04 in Cystinosis

Goals for gene therapy in **Cystinosis**

TO PREVENT OR IMPROVE:



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

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

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

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

Everyday burden of illness, and life expectancy
Unmet needs: medications multiple times per day that cause GI discomfort and acid 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*

CYS-201: Investigator-sponsored* Phase 1/2 study in **Cystinosis patients**

CYSTINOSIS PATIENTS

Day -140	Day -70	Day -20	Day -5	Day 0	Day 28	Weeks 6-14 / Months 6, 12, 18 & 24
Discontinue cysteamine eye drops	Mobilize stem cells	Discontinue oral cysteamine	Conditioning	Infuse AVR-RD-04	Post-treatment assessment	Safety & efficacy assessments

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
• Safety • Efficacy	• 6 patients • adults and potentially adolescents 14–17 years old • Using oral and ophthalmic cysteamine	• 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

AVROBIO

* *Sponsored by UCSD*

CYS-201: IND approved by FDA December 2018

First patient planned ~2H 2019

- **USA FDA CBER** Pre-IND meeting
- **Ethics approval**
- USA **FDA IND approval**

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AVRO-RD-03 in Pompe

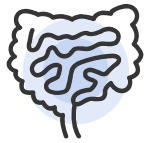
Goals for gene therapy in **Pompe Disease**

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*

AVROBIO

Pompe preclinical program **advancing**

Integrated 3-part solution

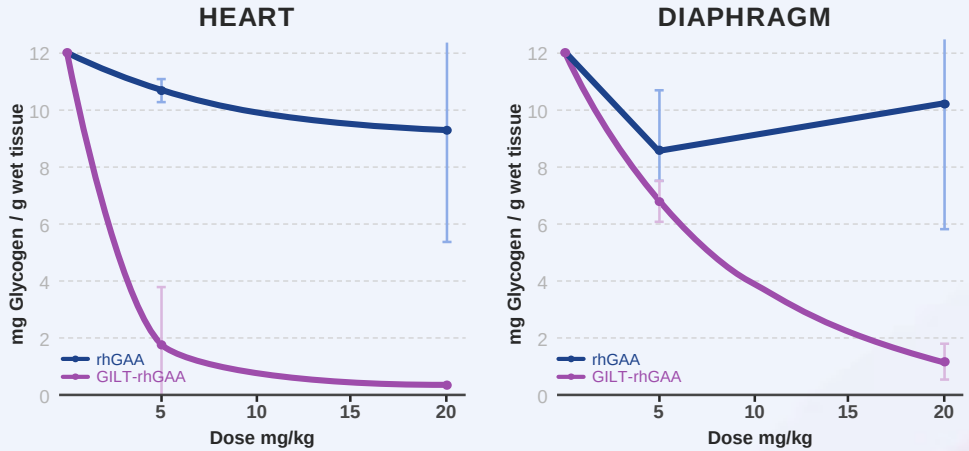
THE CHALLENGE

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

AVROBIO's SOLUTION

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; 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

AVROBIO

Substantial progress on all fronts

Foundation for growth and future commercialization

- Compelling Fabry data across 2 clinical trials
- Gaucher program in clinic 2019
- Cystinosis program in clinic 2019
- **plato**™ in clinic 2019
- Pompe preclinical program advancing
- Strengthened leadership team

AVROBIO

Multiple 2019 milestones anticipated

FABRY

- Phase 1 recruitment set to complete 1H 2019
- FAB-201 Phase 2 trial continues recruitment
- **plato™** incorporated in FAB-201 2H 2019
- Additional trial sites to open in Canada and USA
- Additional readouts throughout the year

GAUCHER

- First patient dosed in GAU-201 Phase 1/2 trial 2H 2019
- **plato™** incorporated in GAU-201 2H 2019

CYSTINOSIS

- First patient dosed in CYS-201 Phase 1/2 trial 2H 2019

POMPE

- Preclinical program advances

Momentum in 2019

- **Compelling Fabry data** across 2 clinical trials
- Substantial **platform and pipeline advances**

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