UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): January 13, 2020

AVROBIO, INC.
(Exact name of registrant as specified in its charter)

Delaware	001-38537	81-0710585
(State or other jurisdiction of incorporation)	(Commission File Number)	(I.R.S. Employer Identification No.)

One Kendall Square
Building 300, Suite 201
Cambridge, MA 02139
(Address of principal executive offices, including zip code)

(617) 914-8420
(Registrant's telephone number, including area code)

Not Applicable
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading symbol(s)	Name of each exchange on which registered
Common Stock, $0.0001 par value per share	**AVRO**	**Nasdaq Global Select Market**

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company ☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 7.01 Regulation FD Disclosure.

On January 13, 2020, AVROBIO, Inc. updated its corporate presentation for use in meetings with investors, analysts and others. A copy of the slide presentation is furnished as Exhibit 99.1 to this Current Report on Form 8-K.

The information in this Item 7.01 and Exhibit 99.1 attached hereto is intended to be furnished and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934 (the "Exchange Act") or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933 or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

99.1 AVROBIO, Inc. slide presentation, dated January 13, 2020.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

AVROBIO, INC.

Date: January 13, 2020

By: /s/ Geoff MacKay
Geoff MacKay
President and Chief Executive Officer

Exhibit 99.1

AVROBIO

Freedom from a lifetime of disease

Corporate Presentation

January 2020

Disclaimer

This presentation has been prepared by AVROBIO, Inc. ("AVROBIO") for informational purposes only and not for any other purpose. Certain information contained in this presentation and statements made orally during this presentation relate to or are based on studies, publications, surveys and other data obtained from third-party sources and AVROBIO's own internal estimates and research. While AVROBIO believes these third-party sources to be reliable as of the date of this presentation, it has not independently verified, and AVROBIO makes no representation as to the adequacy, fairness, accuracy or completeness of any information obtained from third-party sources. While AVROBIO believes its internal research is reliable, such research has not been verified by any independent source.

This presentation may contain forward-looking statements made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. These statements may be identified by words and phrases such as "aims," "anticipates," "believes," "could," "designed to," "estimates," "expects," "forecasts", "goal," "intends," "may" "plans," "possible," "potential," "seeks," "will," and variations of these words and phrases or similar expressions that are intended to identify forward-looking statements. These forward-looking statements include, without limitation, statements regarding our business strategy, prospective products and goals, the therapeutic potential of our investigational gene therapies, the design, commencement, enrollment and timing of ongoing or planned clinical trials, clinical trial results, product approvals and regulatory pathways, potential regulatory approvals and the timing thereof, anticipated benefits of our gene therapy platform including potential impact on our commercialization activities, the expected benefits and results of our implementation of the plato platform in our clinical trials and gene therapy programs, the expected safety profile of our investigational gene therapies, timing and likelihood of success, plans and objectives of management for future operations, future results of anticipated products, and the market opportunity for and anticipated commercial activities relating to our investigational gene therapies, and statements regarding the Company's financial and cash position and expected cash reserves. Any such statements in this presentation that are not statements of historical fact may be deemed to be forward-looking statements.

Any forward-looking statements in this presentation are based on AVROBIO's current expectations, estimates and projections about our industry as well as management's current beliefs and expectations of future events only as of today and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to, the risk that any one or more of AVROBIO's investigational gene therapies will not be successfully developed or commercialized, the risk of cessation or delay of any ongoing or planned clinical trials of AVROBIO or our collaborators or of encountering challenges in the enrollment or dosing in such clinical trials, the risk that AVROBIO may not realize the intended benefits of our gene therapy platform, the risk that our investigational gene therapies or procedures in connection with the administration thereof will not have the safety or efficacy profile that we anticipate, the risk that prior results, such as signals of safety, activity or durability of effect, observed from preclinical or clinical trials, will not be replicated or will not continue in ongoing or future studies or trials involving AVROBIO's investigational gene therapies, the risk that we will be unable to obtain and maintain regulatory approvals for our investigational gene therapies, the risk that the size and growth potential of the market for our investigational gene therapies will not materialize as expected, risks associated with our dependence on third-party suppliers and manufacturers, risks regarding the accuracy of our estimates of expenses and future revenue, risks relating to our capital requirements and needs for additional financing, and risks relating to our ability to obtain and maintain intellectual property protection for our investigational gene therapies. For a discussion of these and other risks and uncertainties, and other important factors, any of which could cause AVROBIO's actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in AVROBIO's most recent Quarterly Report on Form 10-Q, as well as discussions of potential risks, uncertainties and other important factors in AVROBIO's subsequent filings with the Securities and Exchange Commission. AVROBIO explicitly disclaims any obligation to update any forward-looking statements except to the extent required by law.

Note regarding trademarks: plato is a trademark of AVROBIO. Other trademarks referenced in this presentation are the property of their respective owners.

AVROBIO'S MISSION:

To cure rare disease in a single dose

Just as enzyme replacement therapies (ERTs) revolutionized the past, gene therapy has the potential to revolutionize the future.

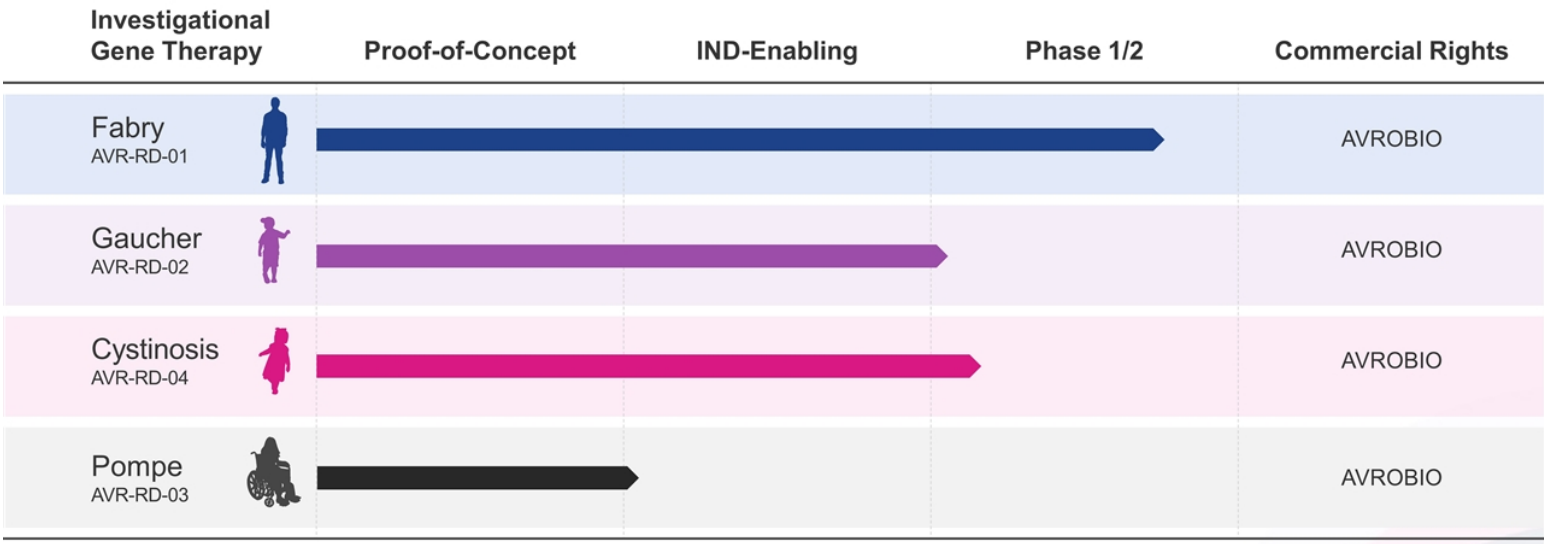
Building value across pipeline and platform

	2019 Accomplishments *…entered 2019 with one program in clinic*	2020 Anticipated Milestones *generating data across 3 clinical programs in 2020…*
FABRY	✓ Reported initial data, including 87% substrate reduction in first kidney biopsy ✓ 9 patients dosed to date	• Continue to report data, including initial plato™ patient data • Gain clarity on potential regulatory approval pathway
GAUCHER	✓ Initiated patient recruitment	• Report initial patient data
CYSTINOSIS	✓ First patient dosed	• Report initial patient data
POMPE	✓ Initiated pre-clinical IND-enabling study	• Complete pre-clinical IND-enabling activities
AVROBIO	✓ Expanded management team ✓ Strengthened balance sheet with $138 million follow-on offering	• Hold first AVROBIO "R&D Day"
plato™	✓ Rolled out plato™ platform ✓ Dosed first patient under plato™ platform	• Dose patients across Fabry and Gaucher trials under plato™ platform • Manufacture on 3 continents

IND: Investigational New Drug

Steady stream of clinical programs

10 patients dosed; 3 programs actively recruiting



Addressing multi-billion dollar markets

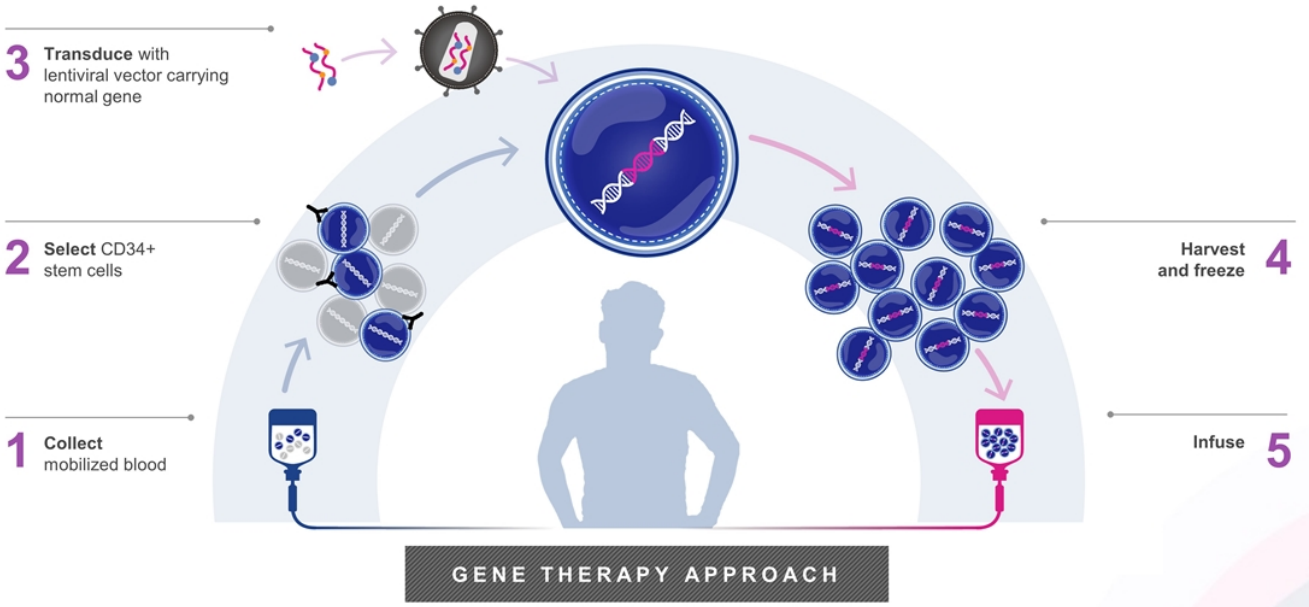
CURRENT STANDARD OF CARE COSTS

Disease	Est. Cost Per Patient Per Year	Approx. 2018 Net Sales	Selected Companies
Fabry	$320k	$1.4B	SANOFI GENZYME, Takeda Shire
Gaucher	$250k-400k	$1.4B	SANOFI GENZYME, Takeda Shire
Pompe	$500k	$1B	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; 2018 Net Sales from company annual and other reports*
**= for Horizon's Procysbi oral therapy (delayed release cysteamine bitartrate)*

Note: Shire acquired by Takeda in 2019

One approach applied across our portfolio



GENE THERAPY APPROACH

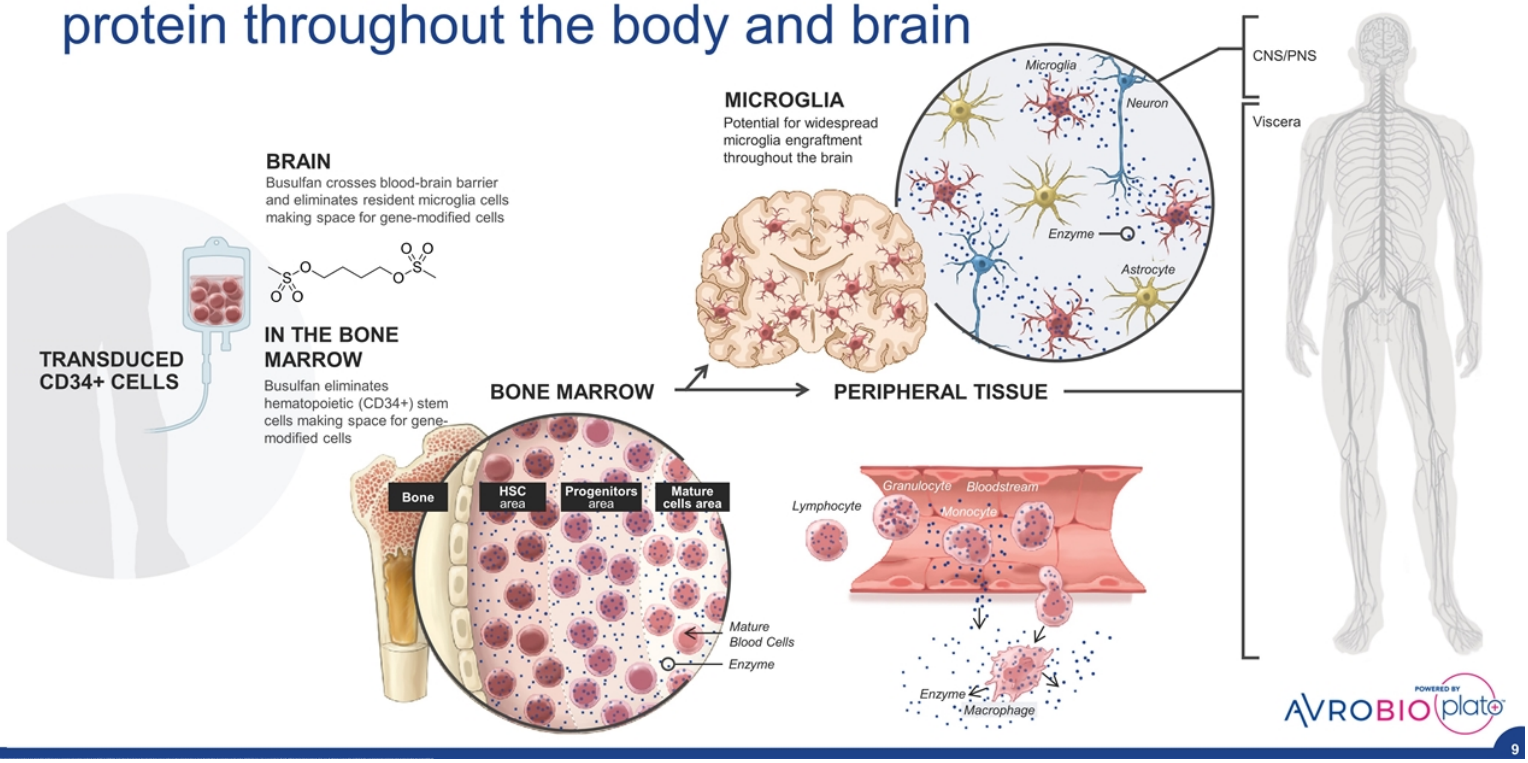
plato™

AVROBIO's foundation for worldwide commercialization

Beginning-to-end manufacturing platform

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

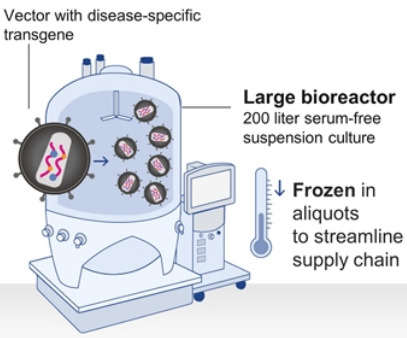
plato™ designed to durably express functional protein throughout the body and brain



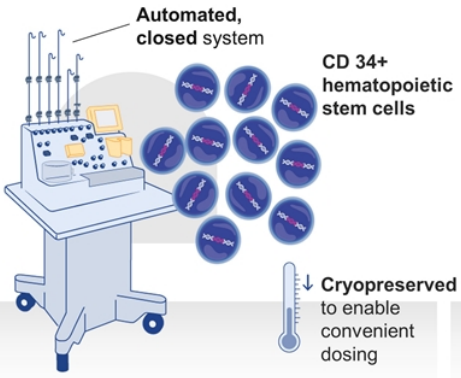
plato™ designed to deliver differentiated, cost-effective approach to large-scale gene therapy manufacturing

1 Vector production → **2 Drug product production** → **3 Scalable, global production suites**

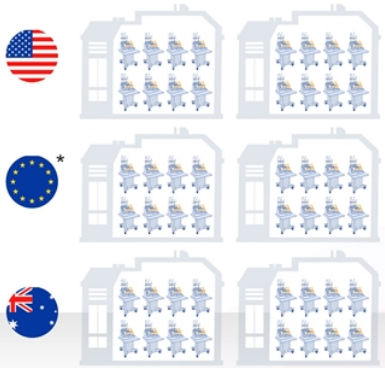
HIGH VOLUME / TITRE



OPTIMIZE STEM CELL QUALITY



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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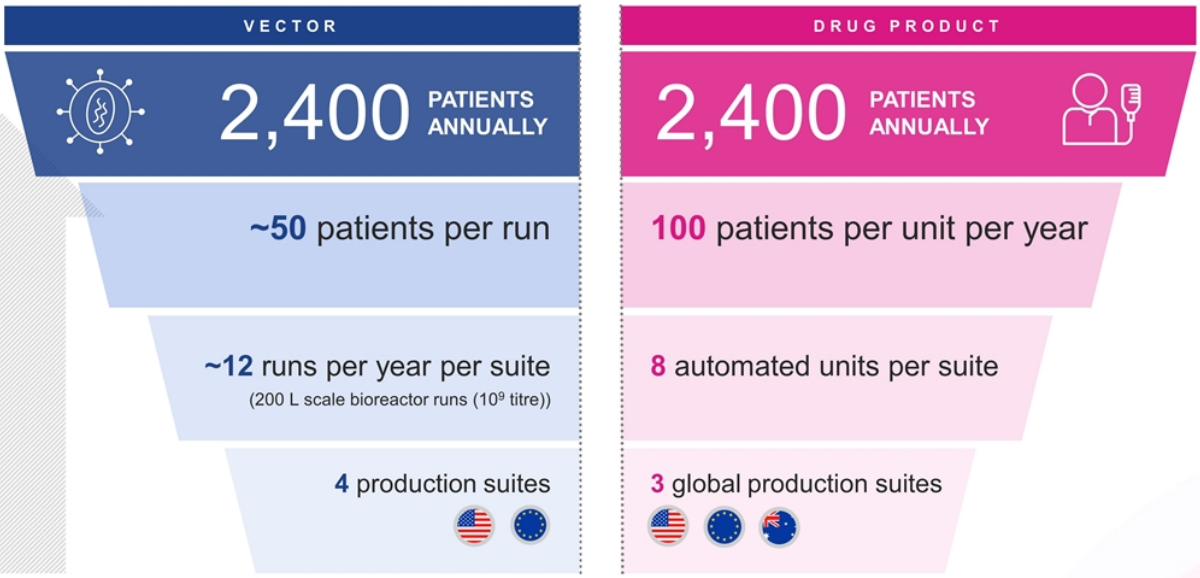
Poised to manufacture at commercial scale



Illustrative

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 Objective

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

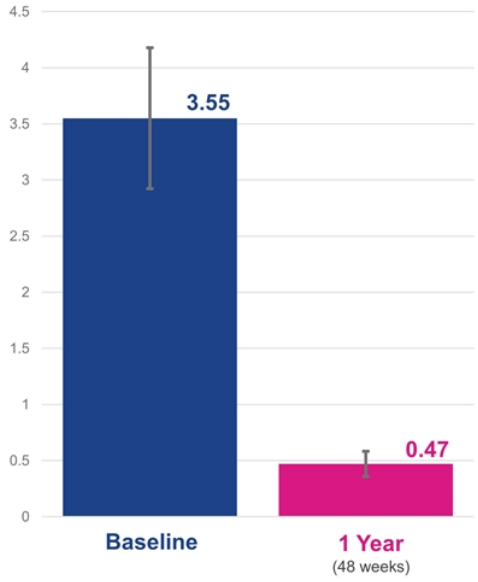
Key Objectives

Safety and efficacy

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

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

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

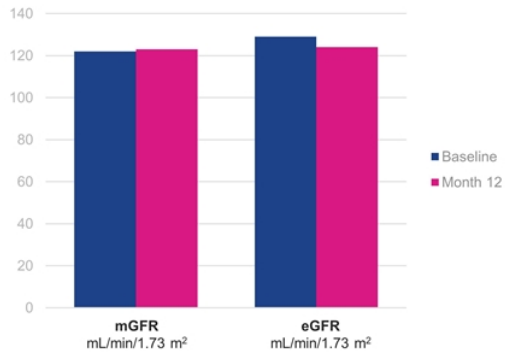


- *Unpaired t test for difference between n=55 PTCs at baseline vs. n=101 PTCs at 1 year; p < 0.0001*
- *Error bar represents the standard deviation*

Baseline: *The last available, non-missing observation prior to AVR-RD-01 infusion*
Note: *With respect to Fabry disease, Gb3 inclusions per PTC is interchangeable with GL-3 inclusions per KIC*
FAB-201-1: *First patient in FAB-201 clinical trial*

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

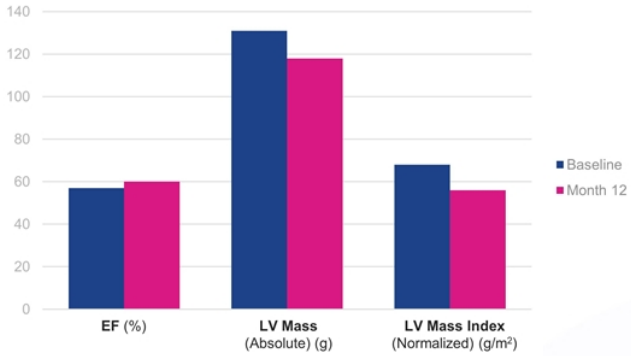
KIDNEY FUNCTION
remains within normal range



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

Source: https://www.kidney.org/atoz/content/gfr
Note: *mGFR is measured Glomerular Filtration Rate; eGFR is estimated Glomerular Filtration Rate*

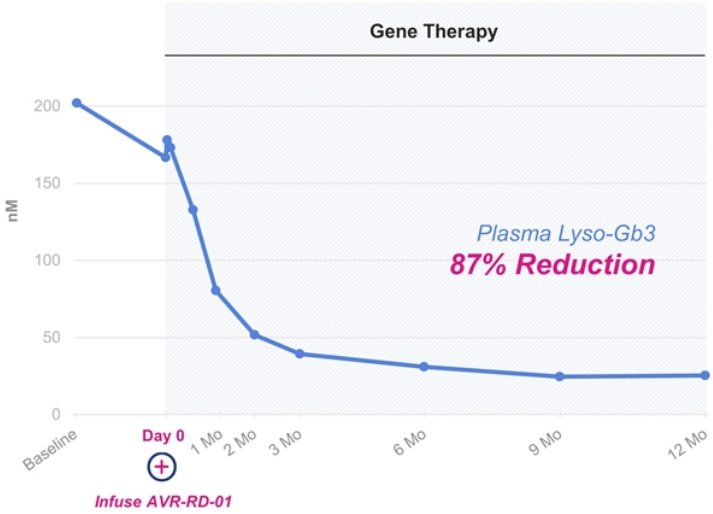
CARDIAC FUNCTION
remains within normal range

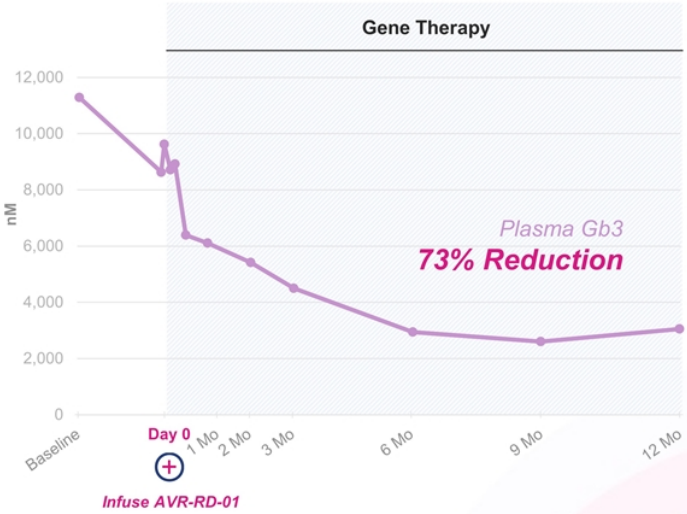


Reference Range Mean Values ± SD	EF (%)	LVM (g)	LVMI (g/m²)
Male (20-39 years)	64.3 ± 4.2	138.9 ± 24.5	67.8 ± 10.7

Source: *Alfakih K et al, J Magn Reson Imaging, 2003*
Note: *EF is Ejection Fraction; LVMI is Left Ventricular Mass Index*

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





Baseline: *The last available, non-missing observation prior to AVR-RD-01 infusion*
Note: *AVR-RD-01 is an investigational gene therapy*

FAB-201

No unexpected trends or safety events identified

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

AEs and SAEs reported

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

Anti-AGA antibodies

- Transient low titer in 1 subject (resolved)

Note: *Safety database cut as of July 10, 2019 for the first 3 patients dosed*

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 Objective

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PHASE 2
AVRO – FAB-201 Trial

Patients

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

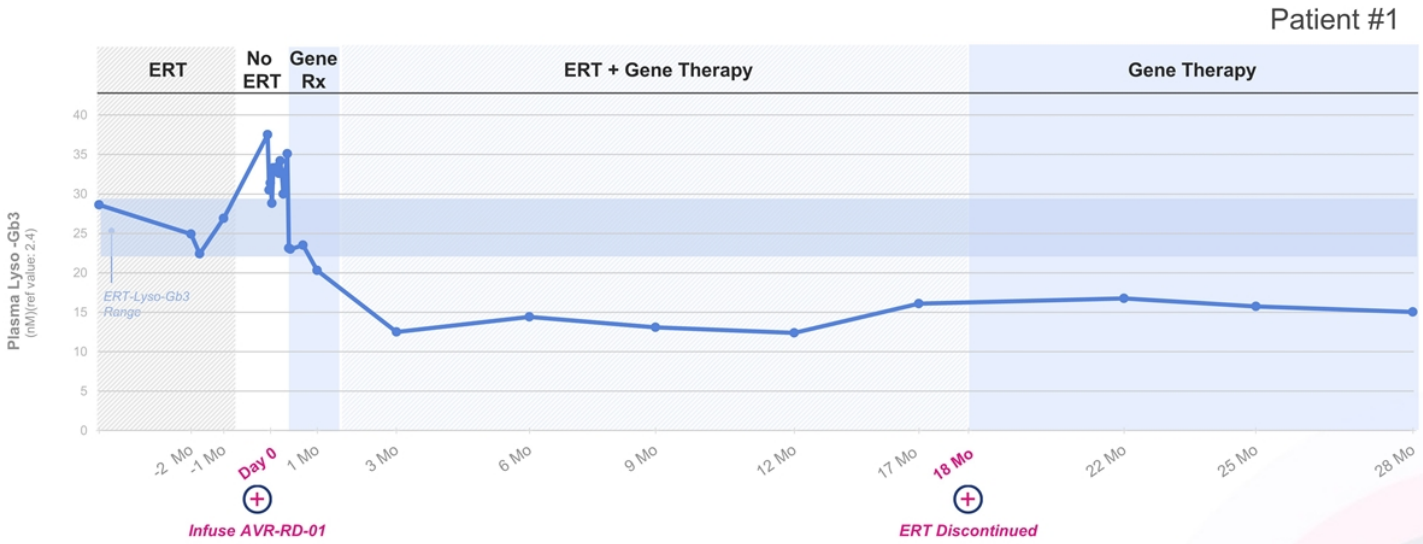
Key Objectives

Safety and efficacy

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

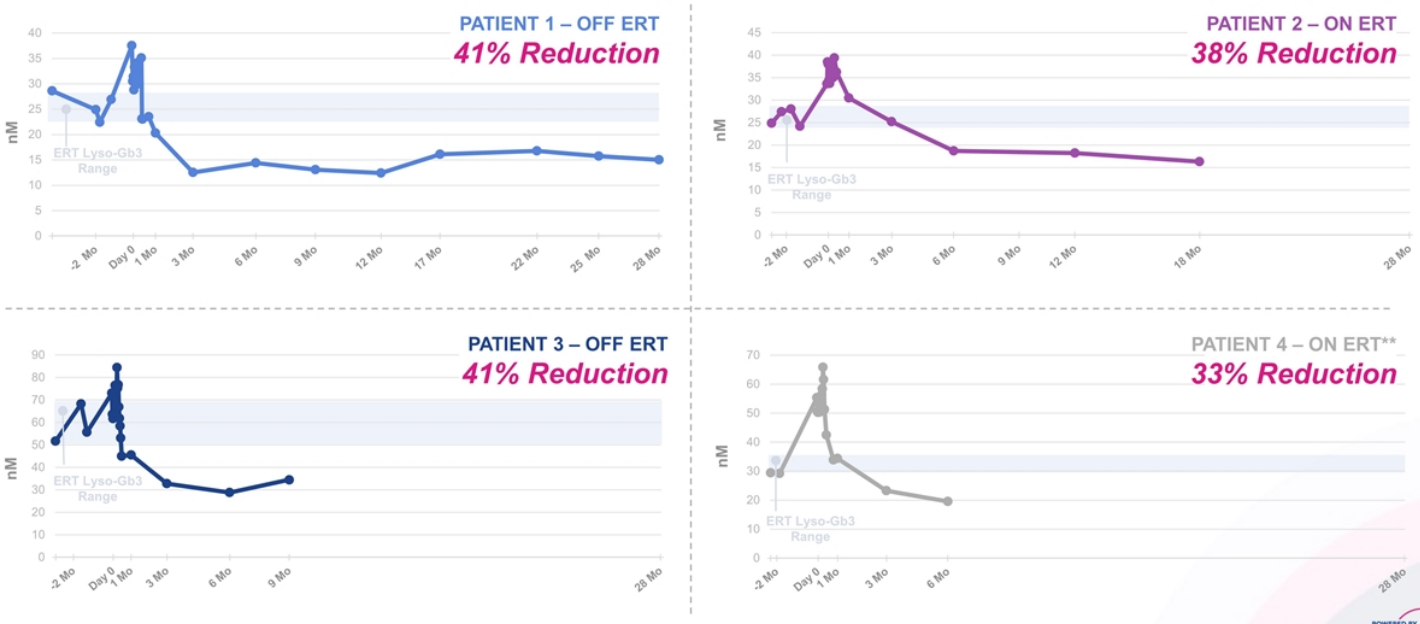
Phase 1: Plasma lyso-Gb3 reduction sustained >2 yrs

Reduced 41% from ERT baseline*



__Baseline:__ The mean of the values reported prior to initiating mobilization
__Note:__ AVR-RD-01 is an investigational gene therapy candidate

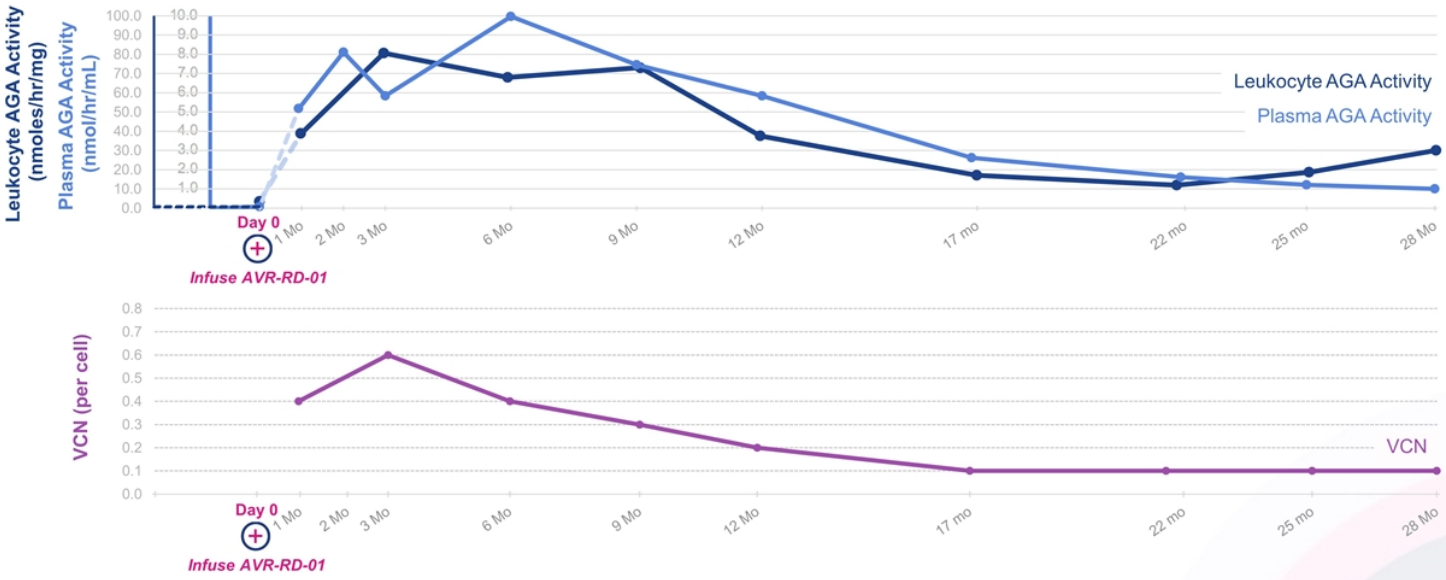
Phase 1: Plasma lyso-Gb3 consistently reduced by 33-41% vs. baseline* ERT at 6+ months post AVR-RD-01 treatment



__Baseline:__ The mean of the values reported prior to initiating mobilization
__Percent reduction:__ As measured from baseline to last assessment
***Patient 4 discontinued ERT 7 months after gene therapy dose*

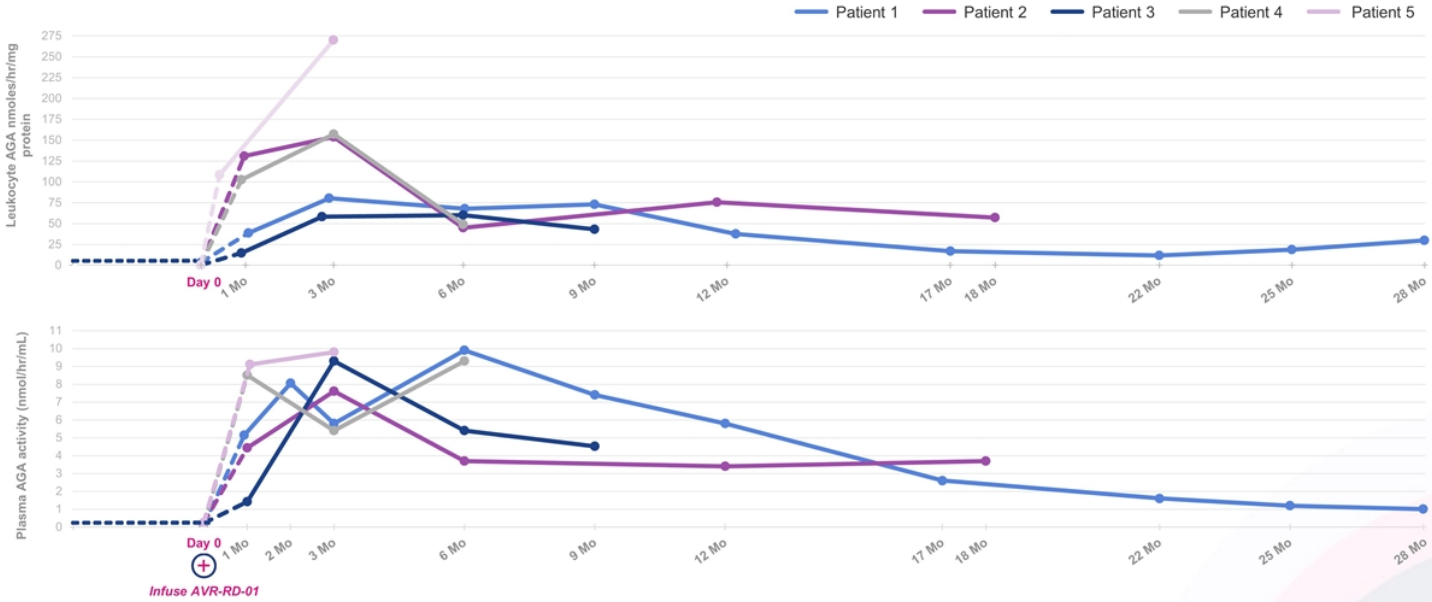
Phase 1: Leukocyte and plasma enzyme activity sustained >2 years; VCN stable

Patient #1



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

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



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

Investigator-sponsored* Phase 1/2 study in Cystinosis

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

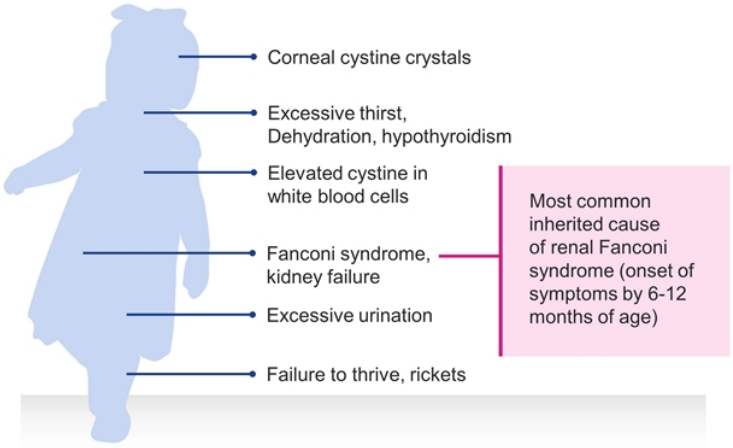
OBJECTIVES	PATIENTS	ASSESS
• Safety • Efficacy	• 6 patients • Adults and potentially adolescents 14–17 years old • Using oral and ophthalmic cysteamine	• Cystine levels in granulocytes • Vector Copy Number (VCN) • Chimerism • Renal, respiratory and endocrine function, ophthalmologic findings, muscle strength, growth, bone density, neurologic and psychometric measures • Safety

* *Sponsored by UCSD*

Cystinosis is a serious, underserved rare disease

Overall incidence ranges from 1 in 100,000 to 200,000 newborns

2,000 patients estimated worldwide;
500 to 600 patients estimated in the U.S.



Current treatment options are inadequate, burdensome, and treat symptoms only

Standard of care cysteamine (a cystine-depleting therapy) delays, but does not halt, progression to end-stage renal disease

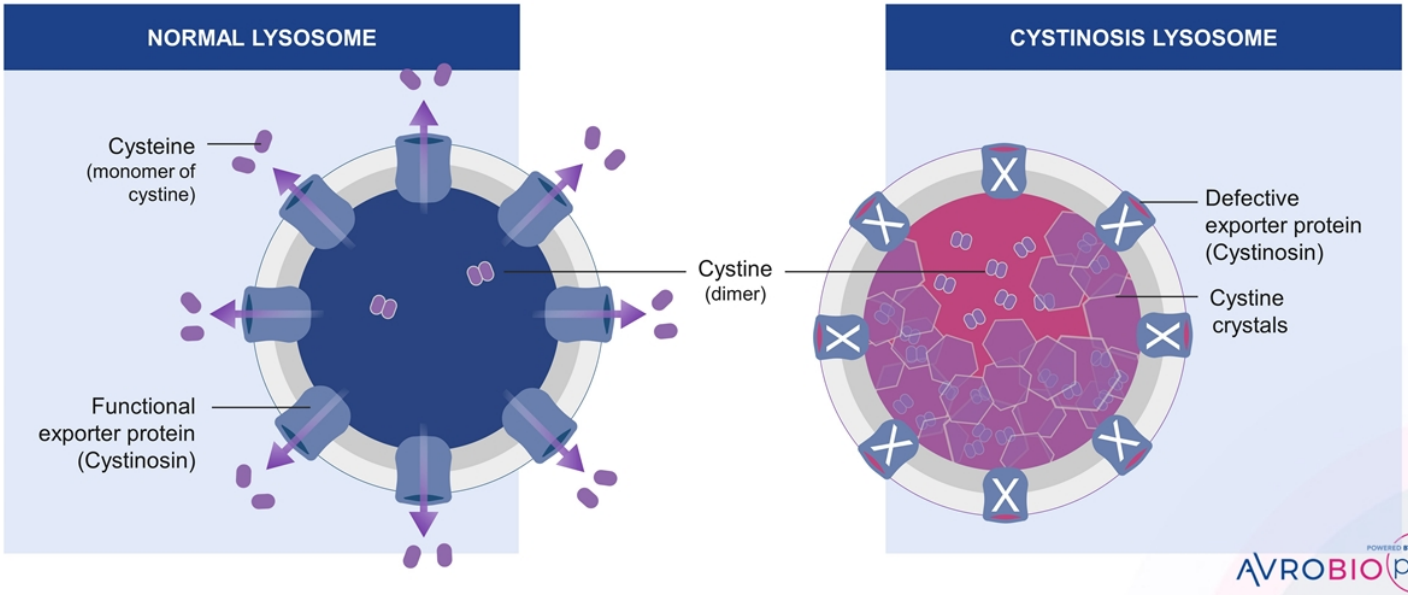
Significant compliance challenges due to:

- Frequency of dosing (Cystagon 4x per day, Procysbi 2x per day, hourly eye drops)
- Side effects, including bad breath and skin odor

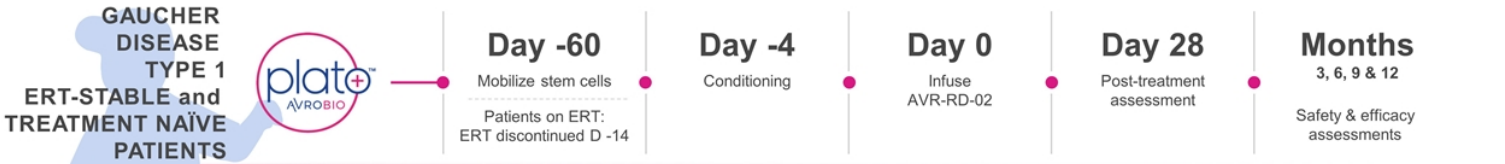
Sources: Wilmer MJ et al, Pediatr Nephrol, 2011, National Institutes of Health, Horizon Therapeutics plc

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

Causes cystine crystals to build up in lysosomes, leading to tissue and organ damage



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



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

OBJECTIVES	PATIENTS	ASSESS
• Safety • Engraftment • Efficacy (functional endpoints and biomarkers) • Evaluate need for ERT re-initiation	• 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

POWERED BY AVROBIO plato

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

KOLs cite numerous unmet needs in Gaucher Type 1, including bone pain/crisis

Skeletal Manifestations

Risk of bone crisis | *Persistent bone pain despite ERT*

- **Bone crises are particularly concerning and can occur despite ERT/SRT**, particularly among patients who have previously had a bone crisis
- Furthermore, **bone pain often persists long after ERT initiation** and can impact quality of life

"Bone pain is the single most intractable manifestation of GD1, and some patients even develop osteonecrosis or fractures."

– KOL

Long-term Complications

Risk of Parkinson's Disease | *Risk of Neurological Abnormalities*

- **Increased risk of Parkinson's Disease** or Parkinson-like symptoms has emerged as a clear unmet need in GD1
- Additionally, GD1 patients are at **increased risk of neurological issues** beyond Parkinson's Disease, such as cognitive decline and neuropathy

"ERT has extended lifespan, but now we are seeing complications that arise over time, like Parkinson's."

– KOL

Treatment Burden

Need for inconvenient, lifelong treatment | *Ongoing cost to healthcare system*

- **Chronic treatment is often burdensome** for GD1 patients, requiring infusions every two weeks and/or close compliance with oral SRTs throughout life
- Additionally, there are **substantial healthcare costs** associated with treating GD1, given cost of chronic ERT

"A one-time treatment could be easier on the patient and might also even help address cost concerns."

– KOL

Sources: *KOL Interviews; market research*

KOL: Key Opinion Leader; SRT: Substrate Reduction Therapy

Gaucher Type 1 patients on SOC have significant unmet needs and are concerned about disease progression

Key Takeaways from Patient Focus Group

"There are days when I can't get up from my chair.

I must speak to customers on the phone instead of visiting them in person."

– 38 year old living with GD1

- Bone pain and fatigue are the most debilitating unmet needs
- Patients have a real fear of developing GBA-Parkinson's
- Some patients have experienced significant GI issues (potentially a neurologic manifestation)
- If gene therapy can address these factors, patients could gain significant quality-of-life back

AVROBIO Gaucher Patient Focus Group
November 16, 2019

Physicians and Patients Answer Differently When Asked About Unmet Needs

Physician	Patient
1. CBC: 8.2*	**1. Fatigue: 7.6***
2. Bone pain: 8.0	2. Bone pain: 7.3
3. Liver/spleen volume: 7.4	3. Liver/spleen volume: 6.4
4. Bone density: 6.9	4. Bone density: 6.2
5. Biomarkers: 6.4	5. Biomarkers: 4.8
6. Fatigue: 5.8	6. CBC: 4.0

**It is noted that physicians ascribed greater importance to testing of Hgb/platelets than did patients. Anemia is both correctable and can be measured objectively and is a cause of fatigue.*

***Sources**: Chen Zion et al. Orphanet J Rare Dis (2016) 11: 53*

GBA-Parkinson's: Glucosylceramidase Beta Parkinson's; CBC: Complete Blood Count; GI: Gastrointestinal; Hgb: Hemoglobin

Milestones anticipated across the pipeline in 2020

FABRY

- Continued recruitment in FAB-201
- Next data release to be held at WORLD*Symposium* in February

GAUCHER

- Enroll first patient in GAU-201 in Q1 2020 with dosing in Q2 2020
- Report initial patient data

CYSTINOSIS

- Continued enrollment in investigator-sponsored trial
- Report initial patient data

POMPE

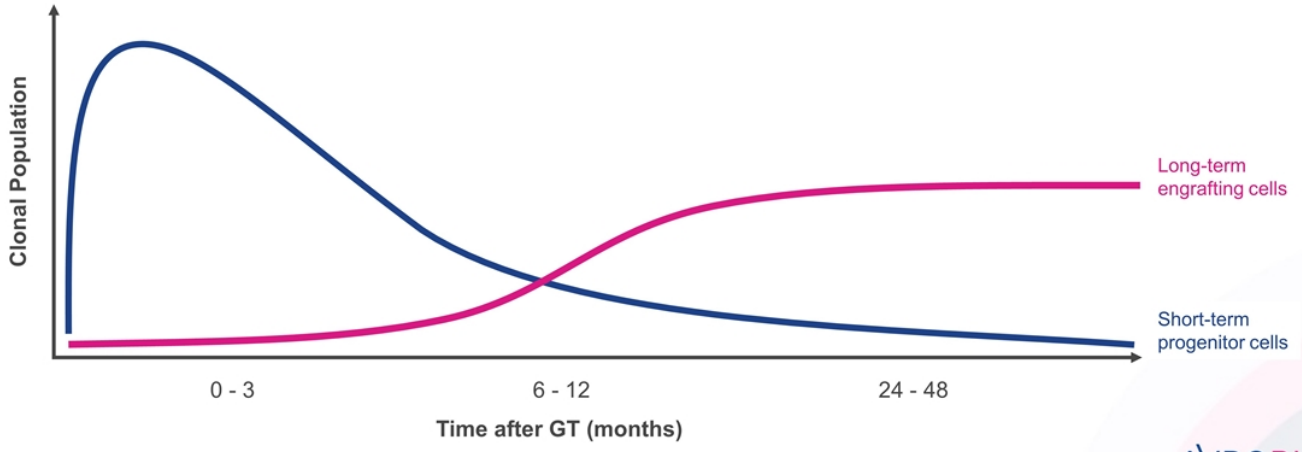
- Complete pre-clinical IND-enabling activities

Appendix

Hematopoietic reconstitution occurs in two distinct phases

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

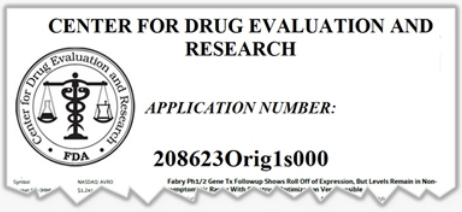
First wave of short-term progenitor cells start to exhaust with progressive takeover by a smaller population of long-term engrafting cells



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

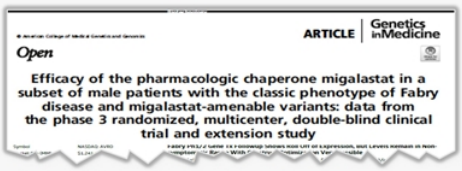
45 Amenable patients* (16 males / 29 females)

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 in Medicine

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[a] 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*