SEEKING GENE THERAPY CURES

August 2024

DISCLAIMER

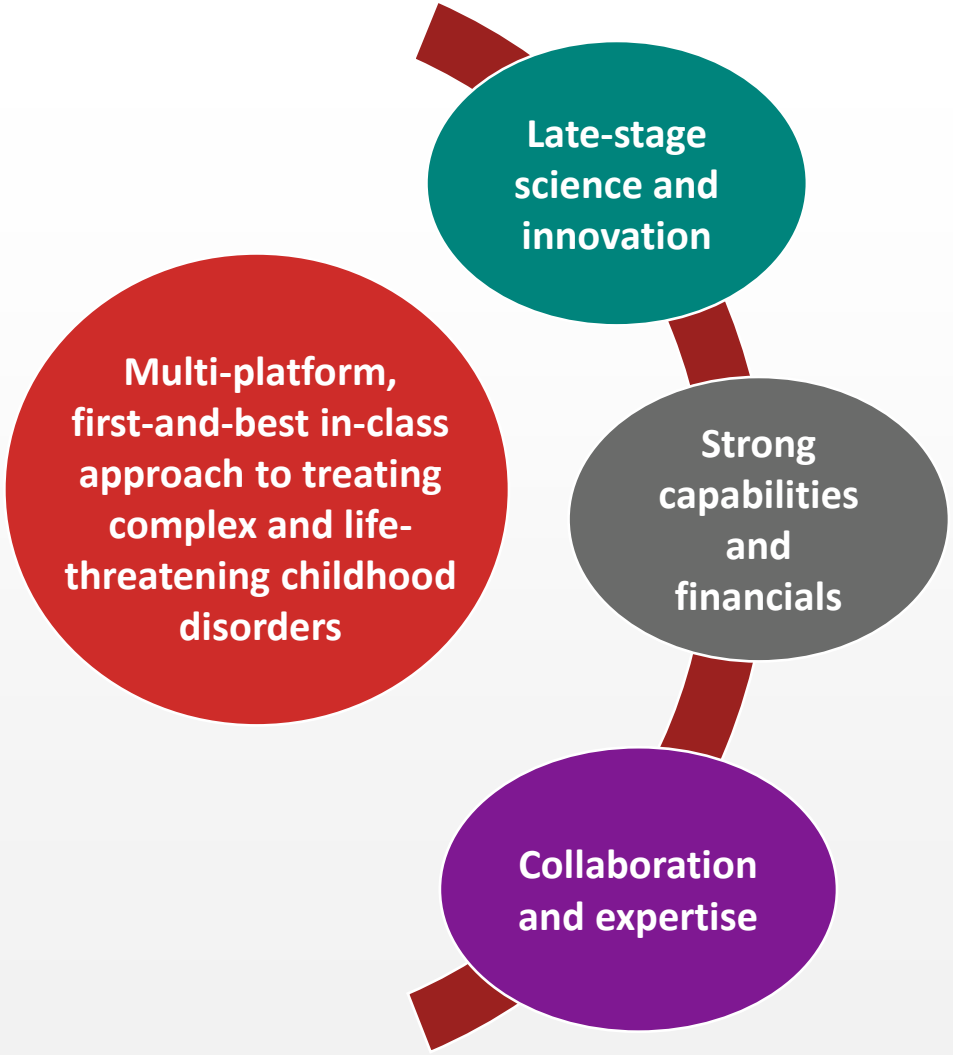
Various statements in this presentation concerning Rocket's future expectations, plans and prospects that involve risks and uncertainties, as well as assumptions that, if they do not materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this release are forward-looking statements. You should not place reliance on these forward-looking statements, which often include words such as "believe," "expect," "anticipate," "intend," "plan," "will give," "estimate," "seek," "will," "may," "suggest" or similar terms, variations of such terms or the negative of those terms. These forward-looking statements include, but are not limited to, statements concerning Rocket's expectations regarding the safety and effectiveness of product candidates that Rocket is developing to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD), Danon Disease (DD) and other diseases, the expected timing and data readouts of Rocket's ongoing and planned clinical trials, the expected timing and outcome of Rocket's regulatory interactions and planned submissions, Rocket's plans for the advancement of its DD program, including its planned pivotal trial, and the safety, effectiveness and timing of related pre-clinical studies and clinical trials, Rocket's ability to establish key collaborations and vendor relationships for its product candidates, Rocket's ability to develop sales and marketing capabilities or enter into agreements with third parties to sell and market its product candidates and Rocket's ability to expand its pipeline to target additional indications that are compatible with its gene therapy technologies. Although Rocket believes that the expectations reflected in the forward-looking statements are reasonable, Rocket cannot guarantee such outcomes. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including, without limitation, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, unexpected expenditures, Rocket's competitors' activities, including decisions as to the timing of competing product launches, pricing and discounting, Rocket's ability to develop, acquire and advance product candidates into, enroll a sufficient number of patients into, and successfully complete, clinical studies, Rocket's ability to acquire additional businesses, form strategic alliances or create joint ventures and its ability to realize the benefit of such acquisitions, alliances or joint ventures, Rocket's ability to obtain and enforce patents to protect its product candidates, and its ability to successfully defend against unforeseen third-party infringement claims, as well as those risks more fully discussed in the section entitled "Risk Factors" in Rocket's Annual Report on Form 10-K for the year ended December 31, 2023, filed February 27, 2024 with the SEC and subsequent filings with the SEC including our Quarterly Reports on Form 10-Q. Accordingly, you should not place undue reliance on these forward-looking statements. All such statements speak only as of the date made, and Rocket undertakes no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

Vision: Seeking Gene Therapy Cures

Mission

To develop **first-in-class** and **best-in-class curative gene therapies** for patients with devastating diseases

A Fully Integrated Gene Therapy Company



Promising top-line clinical data designed to facilitate **US and European registration and launch** with potential for expansion into Asian markets and beyond

Therapeutic area focus:
Heart and bone marrow
Only company with safety and efficacy data for gene therapy targeting the **heart**

~100,000 sq ft

US-based in-house facility dedicated to AAV cGMP manufacturing

Well capitalized to develop full pipeline of assets with approximately

$278.8M[1]

in cash and cash equivalents; **sufficient to fund operations into 2026**

Leadership team with proven track record of

20+

US and ex-US drug approvals and launches

World-class scientific experts, commercial acumen and partners learning from and closely collaborating with **patient communities, HCPs and Payors**

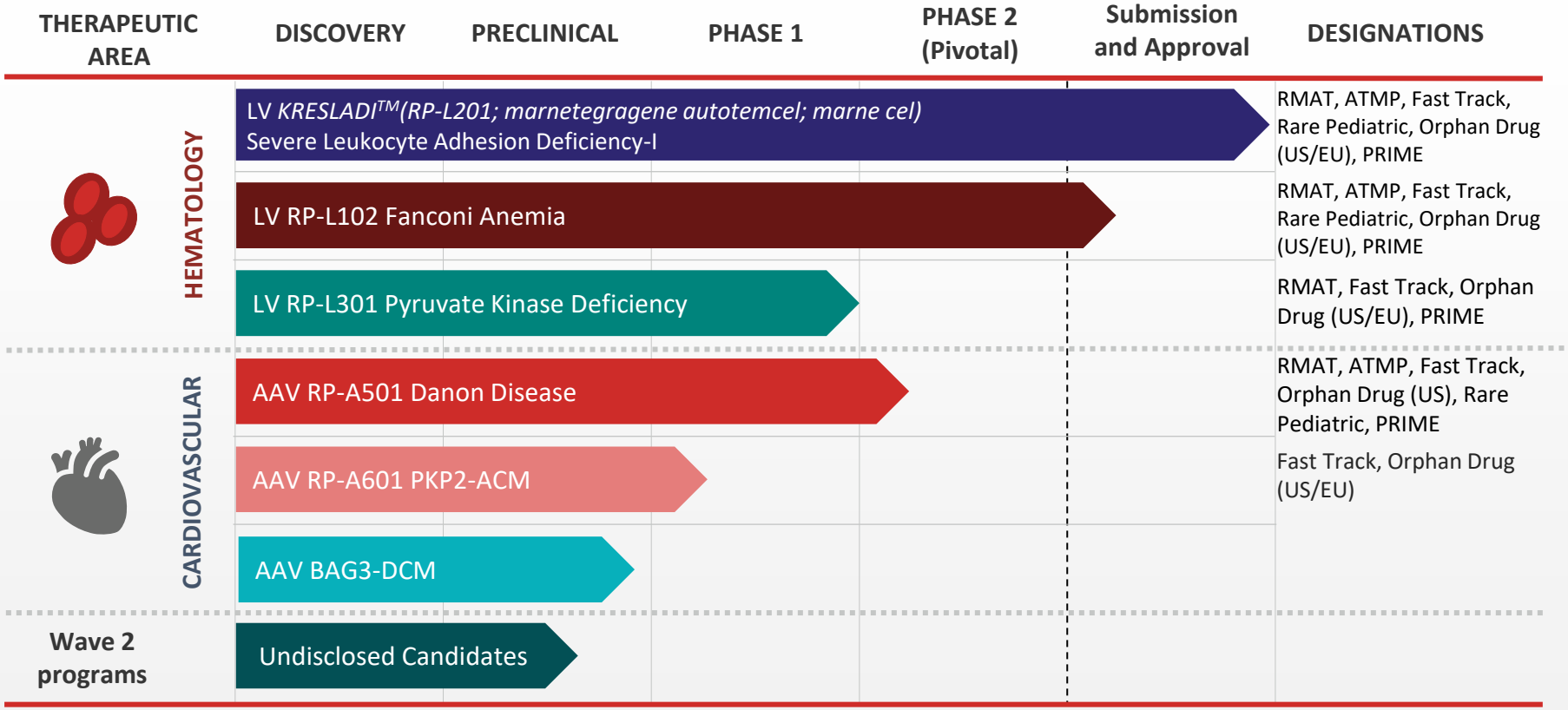
[1]Cash balance as of June 30, 2024.

Strong Science, Carefully-selected Assets and Smart Execution

Criteria used to select programs

- First-, best- and/or only-in-class
- On-target MOA; clear endpoints
- Sizeable market to maximize patient impact

6+ programs with 2 programs fast approaching regulatory filing and launch



AAV, adeno-associated virus; ATMP, advanced therapy medicinal product; BLA, Biologics License Application; LV, lentiviral vector; MAA, Marketing Authorisation Application; MOA, mechanism of action; PRIME, Priority Medicines; RMAT, regenerative medicine advanced therapy. PKP2: plakophilin 2; ACM: Arrhythmogenic Cardiomyopathy; BAG3: BLC2-associated athanogene 3 DCM: Dilated Cardiomyopathy; KRESLAD™, formerly RP-L201; Data on file. Rocket Pharmaceuticals. 2024.

Rocket Offers **Multi-platform Gene Therapy Expertise**

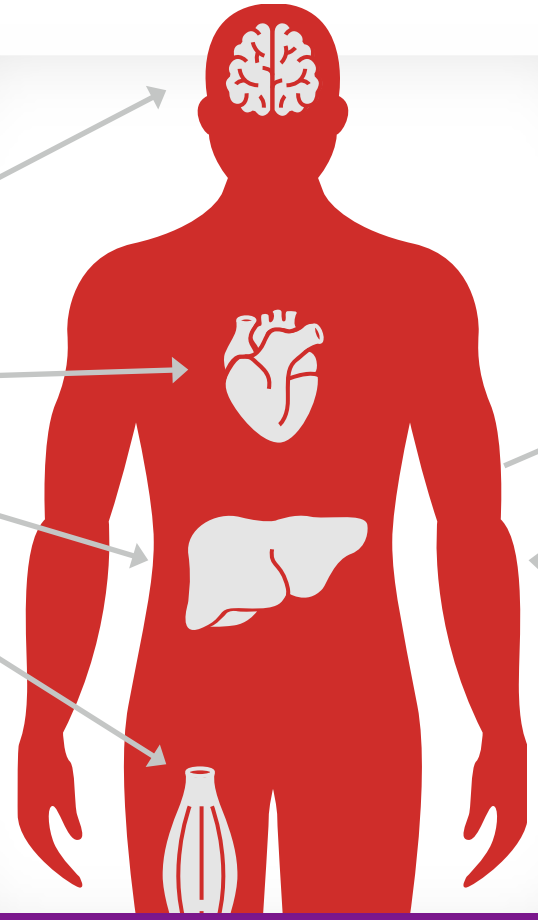
IN VIVO platform

Laboratory-produced AAV

Therapeutic AAV

Direct intravenous injection

RP-A501: Danon Disease
RP-A601: PKP2-ACM
AAV BAG3-DCM



EX VIVO platform

Remove cells and isolate patient HSCs

Laboratory-produced LV

Therapeutic LV

Infusion of modified HSCs

Gene-modified HSCs

RP-L102: Fanconi Anemia
RP-L201: Leukocyte Adhesion Deficiency-I
RP-L301: Pyruvate Kinase Deficiency

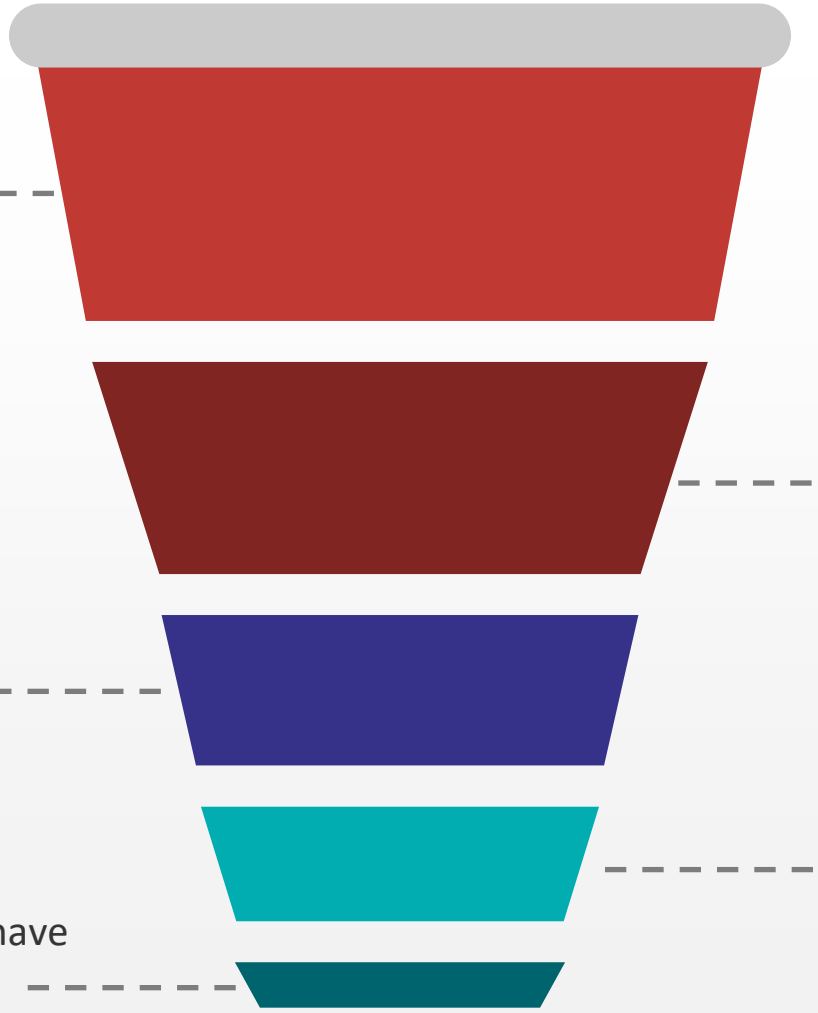
All Rocket therapies transfer full (non-truncated) coding sequence to target tissue

AAV, adeno-associated virus; HSC, hematopoietic stem cell; LV, lentiviral vector; PKP2: plakophilin 2; ACM: Arrhythmogenic Cardiomyopathy BAG3: BLC2-associated athanogene 3 DCM: Dilated Cardiomyopathy.
Data on file. Rocket Pharmaceuticals. 2024.

Rare Diseases Are Associated With a Reduced Lifespan[1]

400 million
people globally are affected by a rare disease[1]

Children account for **50%** **of rare disease patients[1]**



80% **of rare diseases** have monogenic origins[1]

3 of 10 children with a rare disease die before their fifth birthday[1]

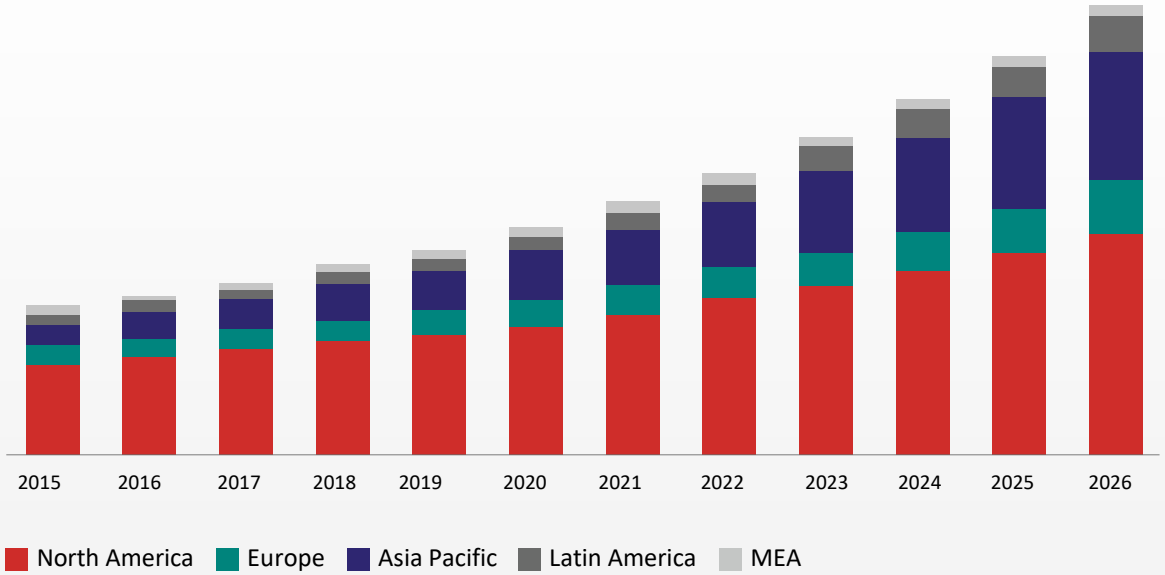
Only about 5% **of rare diseases** have an FDA-approved drug treatment[1]

FDA, Food and Drug Administration.

1. Global Genes. Accessed April 2022. https://globalgenes.org/rare-disease-facts/

Market for Rare Disease Treatment Is **Rising**

Rare disease treatment market by region, 2015-2026 (USD million)[1]



Rare disease treatment market by drug type, 2019 (USD million)[1]

- Rare disease treatment market is projected to grow from **$161.4 billion in 2020** to **$547.5 billion by 2030**[2]
- CAGR of 13.1% projected by 2030[2]

Orphan drug approvals have increased

4-fold[3]

CAGR, compound annual growth rate.

1. Global Market Insights. Accessed April 2022. https://www.gminsights.com/industry-analysis/rare-disease-treatment-market **2.** Global News Wire. Accessed August 2022. https://www.globenewswire.com/en/news-release/2021/02/24/2181634/0/en/Global-Rare-Disease-Market-is-estimated-to-be-US-547-5-billion-by-2030-with-a-CAGR-of-13-1-during-the-forecast-period-by-PMI.html
3. AHIP. Accessed April 2022. https://www.ahip.org/how-big-pharma-makes-big-profits-on-orphan-drugs (increase from 1998 to 2017)

Costs Associated With Rare Diseases Have Increased Exponentially[1]

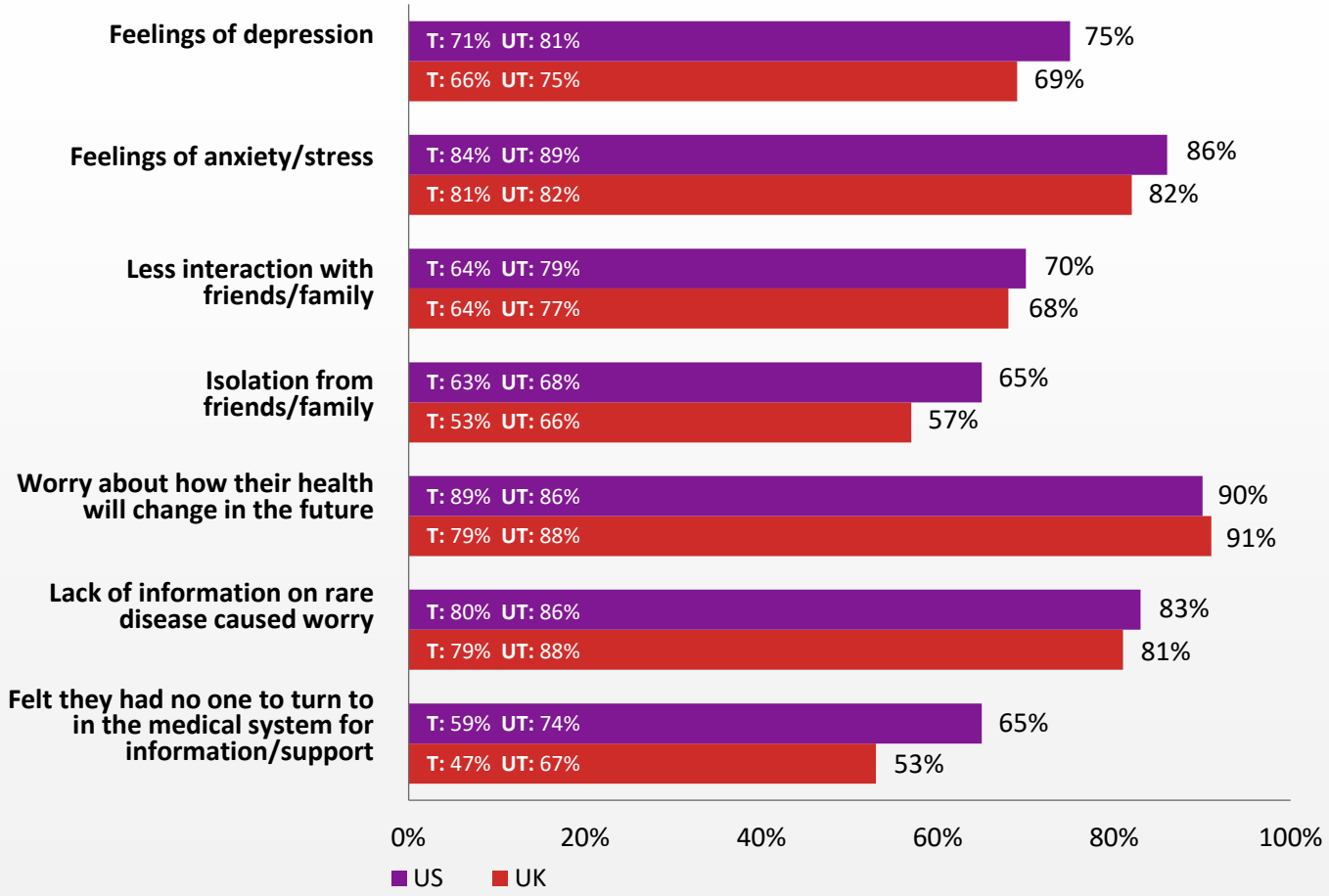
Economic impact[1]

26-fold increase in average per-patient annual cost for orphan drugs* compared to doubled costs for specialty and traditional drugs[1]

Patients with rare diseases or their **caregivers** are **often compelled to leave the workforce**[2]

Cost of **bone marrow** and **heart transplants & maintenance** is high

Emotional impact[4]



*An orphan drug is a pharmaceutical agent developed to treat medical conditions, which, because they are so rare, would not b e profitable to produce without government assistance.
T, treatable; UT, untreatable.

1. AHIP. Accessed April 2022. https://www.ahip.org/news/press-releases/drug-prices-for-rare-diseases-skyrocket-while-big-pharma-makes-record-profits (increase from 1998 to 2017)
2. Every Life Foundation for Rare Diseases. Accessed April 2022. https://everylifefoundation.org/wpcontent/uploads/2021/02/The_National_Economic_Burden_of_Rare_Disease_Study_Summary_Report_February_2021.pdf
3. Global Genes. Accessed April 2022. https://globalgenes.org/wp-content/uploads/2013/04/ShireReport-1.pdf

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Danon Disease (DD): Serious Condition with High Unmet Medical Need

Market Opportunity – US and EU
Prevalence of **15,000 to 30,000** individuals
Annual incidence of **800 to 1,200** individuals

Disease Etiology

- X-linked, dominant, monogenic disease
- Loss-of-function mutations in *LAMP2*

Therapeutic Challenges

- Standard of care:
  - Heart transplant (HTx)
- Limitations:
  - Considerable morbidity and mortality
  - Only ~20% of patients receive HTx
  - Not curative of extracardiac disease

Clinical Manifestations

Impaired autophagy

- Prominent autophagic vacuoles
- Myocardial disarray

Other clinical manifestations

- Skeletal myopathy
- CNS manifestations
- Ophthalmologic manifestations

Severe cardiomyopathy

- Mortality secondary to heart failure or arrhythmia
- Males: Aggressive disease course, median overall survival: 19 years
- Females: Delayed median presentation (~20 years later) due to additional X chromosome, highly morbid and fatal disorder

CNS, central nervous system; *LAMP-2B*, lysosome-associated membrane protein 2B.

Boucek D, Jirikowic J, Taylor M. Natural history of Danon disease. Genet Med. 2011;13(6):563-568.
Brambatti M, Caspi O, Maolo A, et al. Danon disease: Gender differences in presentation and outcomes. Int J Cardiol. 2019;286:92-98.

Phase 1 Data: Benefit Observed Across All Key Clinical Parameters

Early LAMP2, BNP, TnI changes associated with sustained clinical improvement and guided Phase 2 endpoint selection

Cohort	Patient ID	Follow-up (months)	Myocardial LAMP2 Grade (≤M12)	TnI Δ (≤M12)	BNP Δ (≤M12)	LV mass Δ (g)	LV Mass Index Δ (g/m^2.7)	Max LV Wall Thickness Δ (mm)	NYHA class Δ	KCCQ score Δ
Low dose adult/ adolescent	1001 (17.4 aai)	36	1	-75% (M18)	-36%	311 ⇢ 212	85 ⇢ 57	25 ⇢ 23	II ⇢ II	44 ⇢ 49
	1002 (20.3 aai)	36	3	-79%	-76%	989 ⇢ 511	260 ⇢ 129	64 ⇢ 38	II ⇢ II	64 ⇢ 81
	1005 (18.3 aai)	30	2 (M9)	-57% (M9)	-64% (M9)	438 ⇢ 375	98 ⇢ 76	33 ⇢ 24	II ⇢ I	77 ⇢ 85 (M24)
High dose adult/ adolescent	1006 (21.1 aai)	24	1	-47%	-70%	410 ⇢ 300	90 ⇢ 63	22 ⇢ 18	II ⇢ I	79 ⇢ 82
Low dose pediatric	1008 (12.3 aai)	12	1	-86%	-83%	605 ⇢ 447	140 ⇢ 96	42 ⇢ 39	II ⇢ I	50 ⇢ 82
	1009 (11.7 aai)	6	1	-90%	-62%	234 ⇢ 185	83 ⇢ 63	20 ⇢ 20	II ⇢ I	52 ⇢ 78

All specified parameters either improved or stabilized (none deteriorated)

Improved Stabilized Worsened

Does not include pt 1007 in Ph1 trial who had advanced HF with EF<40% at enrollment and received HTx 5M following tx due to pre-existing advanced HF. Patient is currently stable.
aai, age at infusion, BNP, brain natriuretic peptide; hsTnI, high-sensitivity troponin I; KCCQ, Kansas City Cardiomyopathy Questionnaire; LAMP2, lysosome-associated membrane protein 2; LV, left ventricle; NYHA, New York Heart Association. Data cut-off Oct 6, 2022; Grade 0 = negative staining; Grade 1 = ≤25%; Grade 2 =26%-50%; Grade 3 =51%-75%; Grade = 4 >75%. Low dose = 6.7x1013 GC/kg, high dose = 1.1x1014 GC/kg

Latest Phase 1 Pediatric Data Shows Sustained Improvements in Biomarkers, Symptoms, and Function

Subject ID: A501-008-1008

Baseline Characteristics

AGE AT INFUSION
12.3 years

ICD yes[1]

WPW yes

MAX LV WALL THICKNESS
41.9 mm, z-score +32

6MWT
438 meters

18 months

Variable	Baseline[2]	Most Recent Follow-up
LAMP2 protein[3]	0	1 (M12)
Troponin-I (ng/mL)	1.89	0.30 (-84%)
BNP (pg/mL)	1837	328 (-82%)
NYHA Class	II	I
LV Mass Index	140	96 (-31%)[4]

Subject ID: A501-008-1009

Baseline Characteristics

AGE AT INFUSION
11.7 years

ICD no

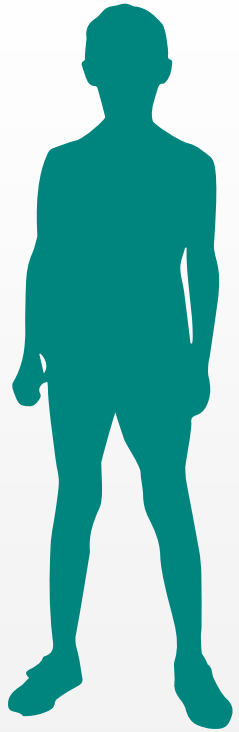
WPW no

MAX LV WALL THICKNESS
19.8 mm, z-score +12

6MWT
553 meters

12 months

Variable	Baseline[2]	Most Recent Follow-up
LAMP2 protein[3]	0	1 (M6)
Troponin-I (ng/mL)	0.67	0.08 (-88%)
BNP (pg/mL)	297	163 (-45%)
NYHA Class	II	I
LV Mass Index	83	60 (-28%)[4]



[1] Recommended prior to enrollment; ICD implanted 3 months after RP-A501 infusion. [2] Baseline values for troponin-I and BNP are the mean values from all pre-dose visits. [3] Extent of LAMP2 expression grading: Grade 0 = negative staining, Grade = 1 < 25%, Grade 2 = 26-50%, Grade 3 = 51-75%, Grade = 4 > 75%;. [4]M12 Note: All data preliminary, not yet validated.
6MWT, 6-minute walk test; BNP, brain natriuretic peptide; ICD, implantable cardioverter defibrillator; KCCQ, Kansas City Cardiomyopathy Questionnaire; LAMP2, lysosome-associated membrane protein 2; LV, left ventricle; NYHA, New York Heart Association; WPW, Wolff-Parkinson-White syndrome.

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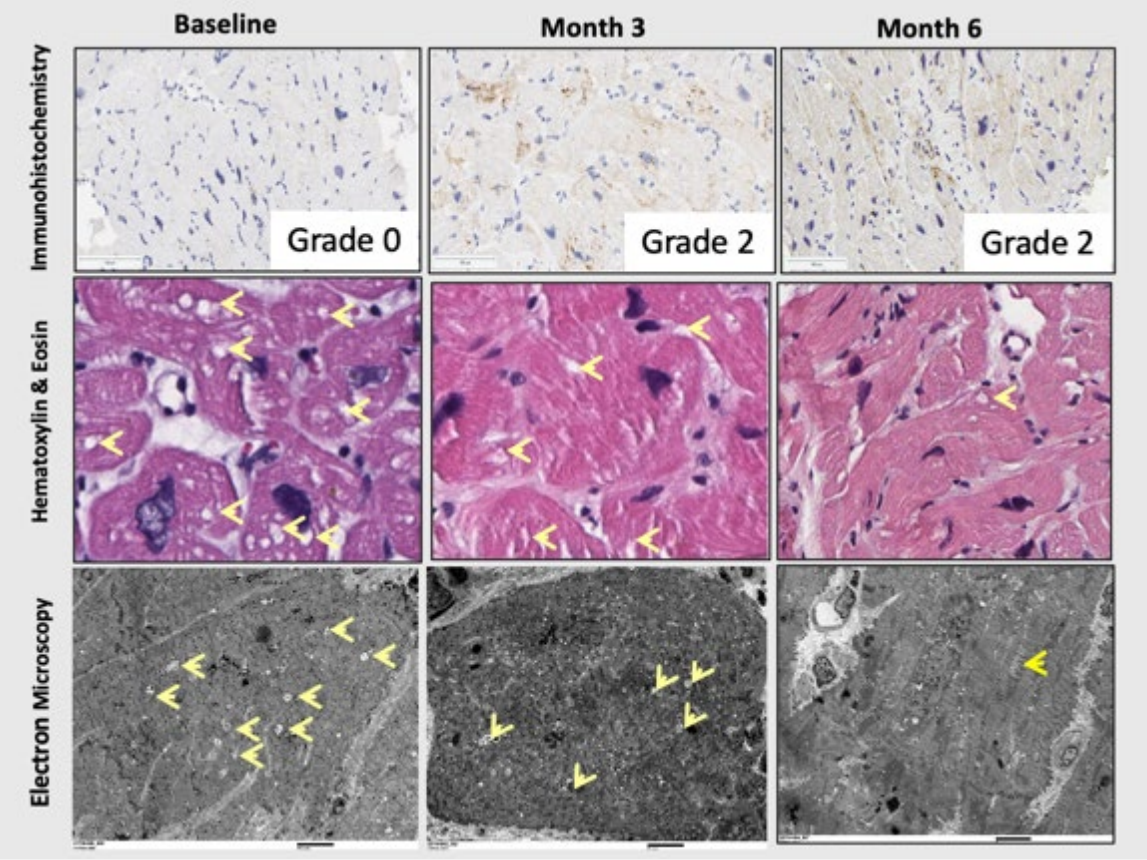
RP-A501 Increases LAMP2 Protein and Decreases Vacuolization

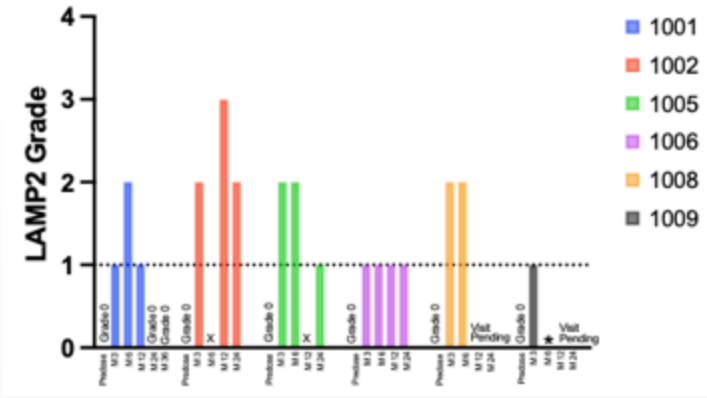
Enhanced autophagy leads to improved myocardial ultrastructure and clinical phenotype

Endomyocardial Biopsy Images

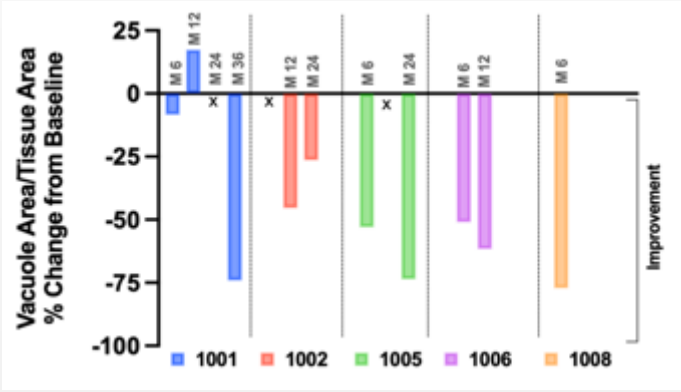
(Subject 1008, RP-A501 Phase 1 Study)



Myocardial LAMP2 Protein Expression



Vacuolar Area of Endomyocardial Tissue



Rocket AHA 2022 Poster Presentation reflects September 27, 2022 data cutoff. LAMP2, lysosome associated membrane protein 2; M, month; IHC = immunohistochemistry.
Grade 0 = negative staining; Grade 1 = ≤25%; Grade 2 = 26%-50%; Grade 3 = 51%-75%; Grade 4 = >75%.

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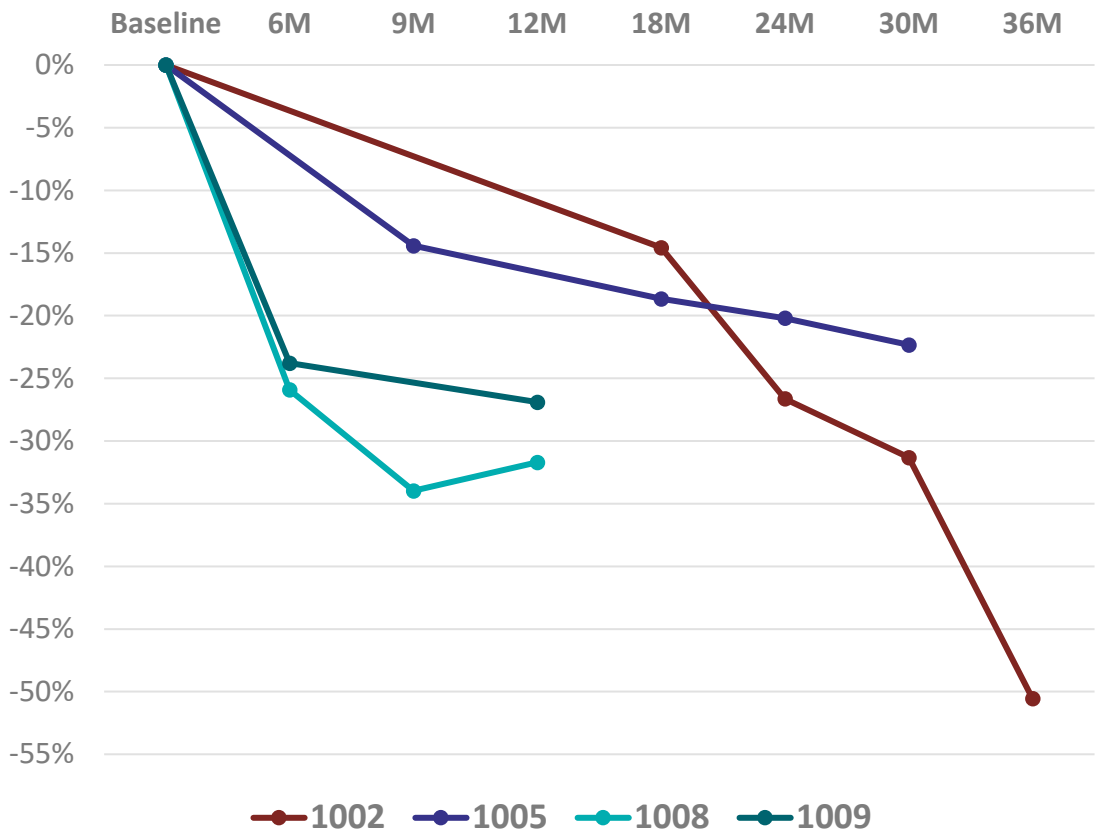
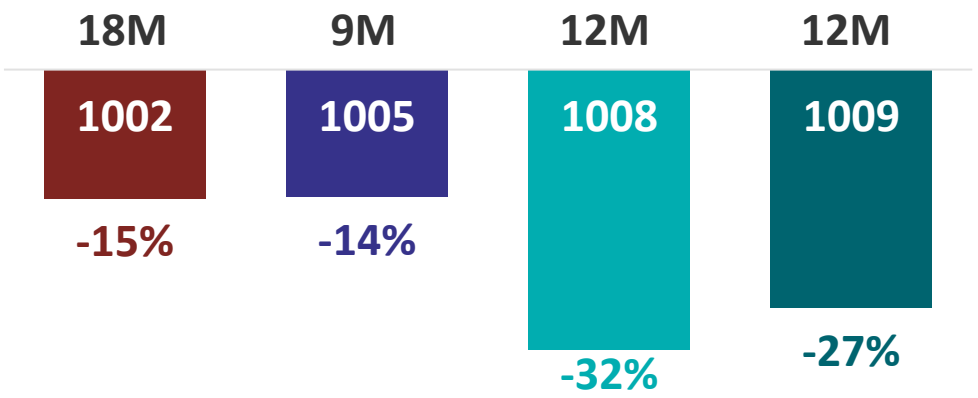
LV Mass Index in RP-A501 Phase 1 Study

RP-A501 Phase 1 Low-Dose Cohort: LV Mass Index % Change from Baseline[1]



RP-A501 Phase 1 Study: LV Mass Index % CFB at ~12M

(9M or 18M where 12M not available)[2]



- >20% LVMI decrease observed as early as 6M in pediatric cohort, sustained to 12M timepoint
- Adult patients with appropriate immunomodulation show >10% LVMI decrease around 12M[2] with further decreases out to 30-36M of 22% to 51%

[1] Does not include patient 1001 (unmonitored immunomodulation). [2] 12M visit data missing for 1002 and 1005 due to pandemic-related travel issues.

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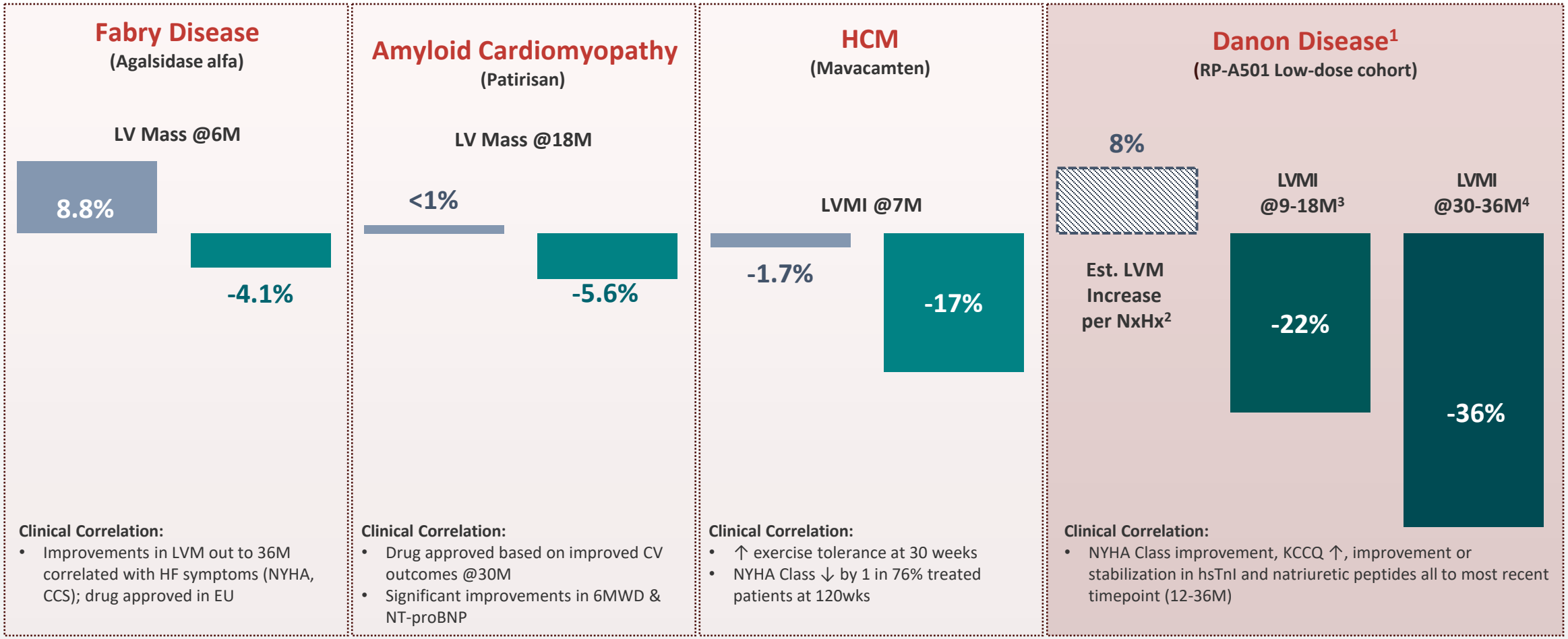
LV Mass / LV Mass Index (LVMI) Improves in DD with RP-A501

LV hypertrophy decreases greater than/comparable to other approved therapies



[1] RP-A501 Phase 1 low-dose cohort data; averages do not include 1001 (unmonitored immunomodulation) and 1006, 1007 (high dose patients). [2] Reflects estimated LV Mass increase over 12-18M for DD patients based on retrospective natural history data-set (shown on slide 20). [3] Reflects average of 1002 18M, 1005 9M, 1008 12M, 1009 12M. [4] Reflects average of 1005 30M, 1002 36M
Hughes 2008. Heart; Solomon 2019. Circulation; Saberi 2021. Circulation

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Insights from Danon Disease Patients Treated on the Phase 1 Trial

He can walk upstairs without being short of breath or having to stop half-way. He doesn't have chest pain or fast heart rates like he used to. Another amazing thing we have seen is about 4 months after his therapy trial he started working and stopped using his motorized scooter altogether. -**Patient 1005**

Prior to therapy, he would say "my wish is not to die young." After gene therapy, we see him smile more because he was able to hold down a steady part-time job and can live independently in an apartment of his own. He is living a life he didn't think would be possible.
–Patient 1006

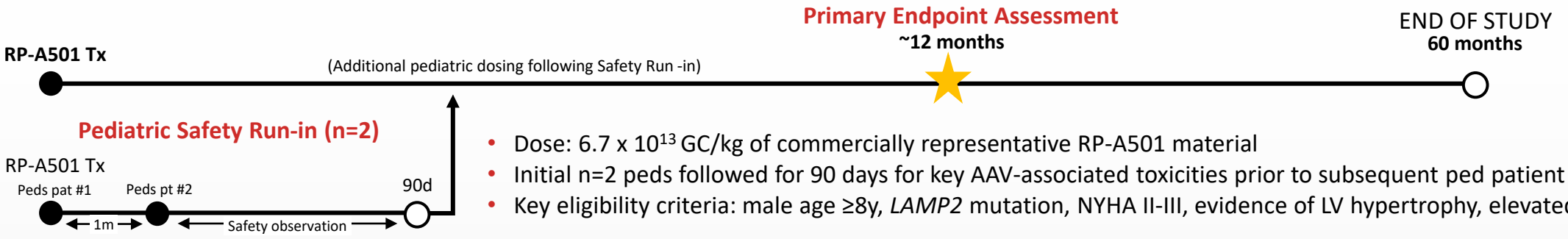
He went to overnight summer camp on his own for the first time and is no longer out of breath walking up stairs.
-Patient 1008

He is now able to exercise on a more regular basis. After treatment, he was able to participate in an organized walk with his father completing most of the 10K course. **-Patient 1009**

Phase 2 Trial Design – 12 Patients with 12-month Primary Endpoint Duration

Pivotal, global, single-arm, open label study

PIVOTAL PHASE 2 STUDY DESIGN



- Dose: 6.7 x 10^{13} GC/kg of commercially representative RP-A501 material
- Initial n=2 peds followed for 90 days for key AAV-associated toxicities prior to subsequent ped patient enrollment
- Key eligibility criteria: male age ≥8y, *LAMP2* mutation, NYHA II-III, evidence of LV hypertrophy, elevated hsTnI

CO-PRIMARY ENDPOINT (AA)

- LAMP2 protein ≥ Grade 1 (IHC) AND
- Left Ventricular Mass (LV Mass): ≥10% ↓

SECONDARY & EXPLORATORY ENDPOINTS

- hs-troponin I (key secondary)
- Natriuretic peptides
- QoL instruments (KCCQ, PedsQL, PGI-C, PGI-S)
- NYHA Class
- 6MWT
- Event free survival
- Treatment emergent safety events
- Actigraphy

RISK MANAGEMENT PLAN, TRIAL OVERSIGHT

- Immunomodulatory regimen of Rituximab, Sirolimus, corticosteroids.
- Clinical monitoring team to closely monitor labs, clinical sequelae for AAV-associated toxicities.
- IDSMC: expertise in adult and pediatric cardiomyopathy, immunology, and biostatistics

CONCURRENT NATURAL HISTORY STUDY

Tx, treatment; LV, left ventricular; NYHA, New York Heart Association; hsTnI, high-sensitivity troponin I; QoL, quality of life; KCCQ, Kansas City Cardiomyopathy Questionnaire; PedsQL, Pediatric Quality of Life Inventory; PGI-C, Patient Global Impression of Change; PGI-S, Patient Global Impression of Severity; IDSMC, Independent Data Safety and Monitoring Committee.

Primary Endpoint Is Reasonably Likely to Predict Clinical Benefit

Justification for use of LAMP2 protein expression and LV Mass

WT Full Length LAMP2 Protein Expression

- Mutation of *LAMP2* is root cause of Danon disease
- Epidemiologic support: even modest levels of LAMP2 confer a 2-decade survival advantage in female patients
- RP-A501 delivers full coding sequence of WT *LAMP2* gene
- Pre-clinical LAMP2 restoration conferred histologic, functional and survival benefits in LAMP2 knock-out model[1]
- Phase 1: LAMP2 expression associated with decreased vacuolar area, improved myofibrillar disarray, clinical improvement

Left Ventricular Mass

- Largest known hearts are Danon disease hearts
- Severity of the cardiomyopathy in Danon disease is the major prognostic factor[2]
- Retrospective natural history shows year-over-year increases in LV mass in Danon disease patients
- Phase 1: Consistent and significant reductions in LV mass as early as 6 months by echocardiography and cardiac MRI

Primary Endpoint Will Be Interpreted in a Clinical Context:

- *All components are measurable and unlikely to improve in the absence of a true treatment effect*
- *Primary endpoint will be assessed in the context of biomarkers, symptoms, QOL, clinical events derived from secondary endpoints and concurrent natural history study*
- *Phase 1 trial: LAMP2 expression and LV Mass improvements seen as early as 6 months in pediatric subjects with updated immunomodulation regimen*

[1] Manso 2020. *Sci Transl Med.;* [2] D'souza 2017. *J Community Hosp Intern Med Perspect*.

In-House Manufacturing to Support Danon Pivotal Study and Commercial Production

- **2 Successful Danon AAV cGMP batches produced in Q4 2022**
- **Superior specifications to Phase I material**; allow for full dosing with lower total viral particles, potentially further improving safety profile
  - *Productivity:* ~3X increase in number of patient treatments per batch
  - *Product Quality:* Significant increase in full versus empty viral particles
  - *Product Comparability:* All attributes tested to date are comparable or improved
- **Regulatory progress and production capacity can support pivotal study and commercialization**
  - FDA clearance on continued utilization of HEK-293 cell-based process through commercial
  - FDA alignment on comparability approach
  - Potency assay developed in-house

Overall, in-house cGMP manufacturing delivers commercial-ready product with higher yield, improved quality, and potential for enhanced safety profile

RP-L102 for Fanconi Anemia Complementation Group A (FA-A)

Fanconi Anemia (A, C, and G)

Market Opportunity – US and EU
Prevalence of **5,500 to 7,000** individuals
Annual incidence of **200 to 275** individuals

Disease etiology

- **FA-A is an autosomal recessive disease caused by *FANCA* gene mutations**
- **FA proteins enable DNA repair**
- **FA-A accounts for 60% to 70% of FA cases**

Therapeutic challenges

Standard of care:
- Allogeneic HSCT

Limitations:
- Significant toxicities, especially for patients who do not have an HLA-identical sibling donor (~80%)
- 100-day mortality
- GvHD
- Increased long-term cancer risk

Clinical manifestations

Disorder of DNA repair characterized by:
- Progressive BMF; 80% of patients experience BMF within first decade of life
- Predisposition to hematologic malignancies and solid tumors

*Gene therapy approach: Selective advantage of corrected cells allows for **ex-vivo LV therapy without conditioning**; highly favorable benefit risk profile*

BMF, bone marrow failure; FA, Fanconi Anemia; FA -A, FA, group A; FANC, FA complementation group; GvHD, graft-versus-host disease; HLA, human leukocyte antigen; HSCT, hematopoietic stem cell transplantation.
Alter BP, Giri N, Savage SA, et al. Malignancies and survival patterns in the National Cancer Institute inherited bone marrow failure syndromes cohort study. Br J Haematol. 2010;150(2):179-188.

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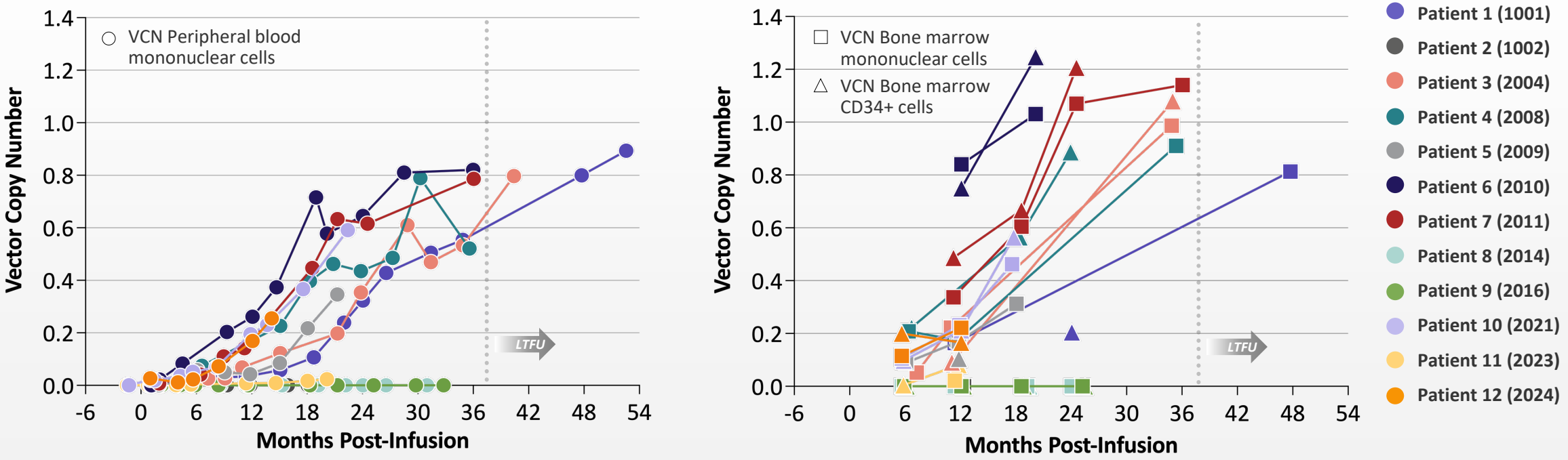
Progressively Increasing and Sustained Genetic Correction in 8 of 12 Patients ≥1 Year Post–RP-L102 in Pivotal Phase 2 Trial

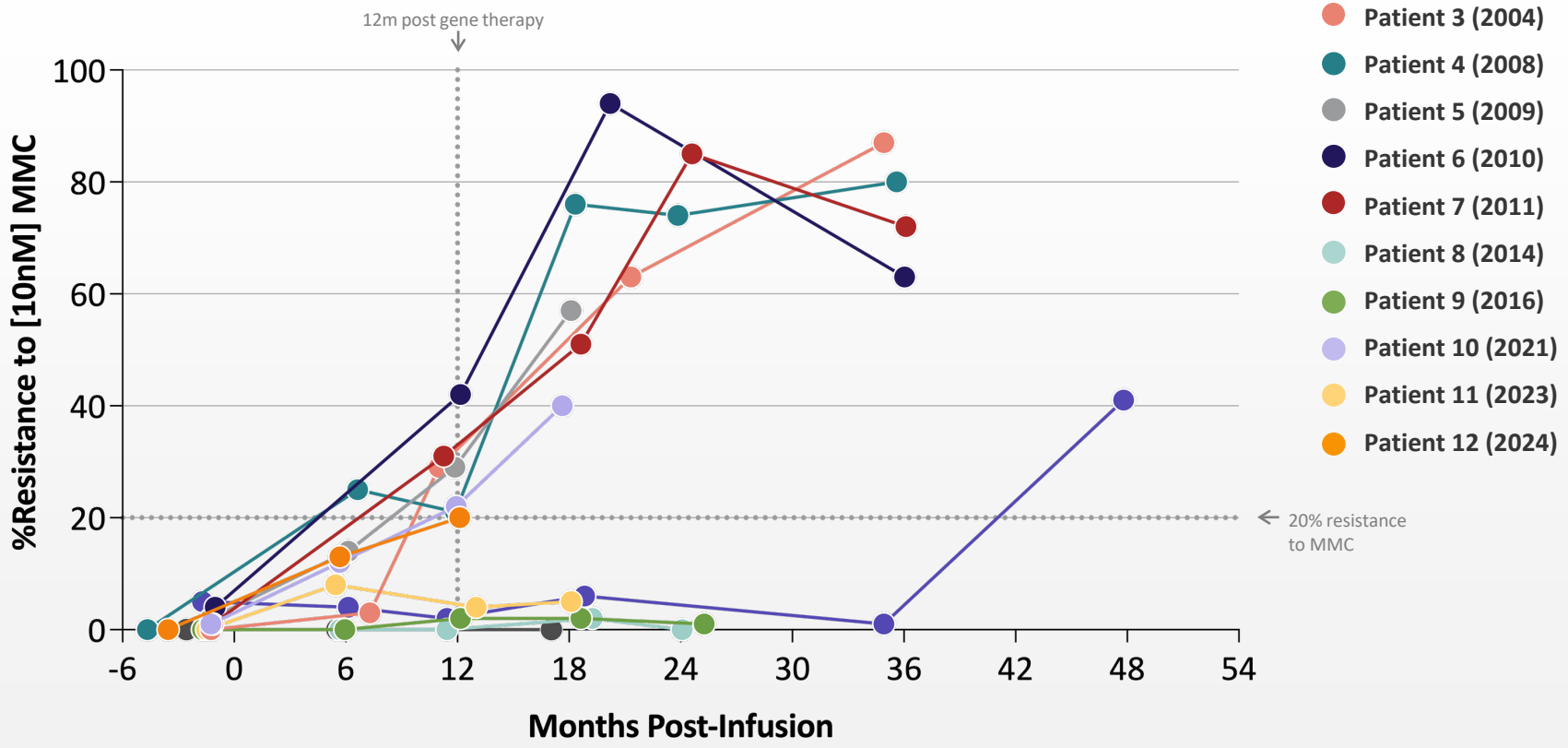
Progressive increases in PB and BM gene marking in 8 patients



BM, bone marrow; PB, peripheral blood; PBMC, PB mononuclear cells; VCN, vector copy number.
BM VCN not available at some stipulated time points due to insufficient sample to perform assay.
Data cut-off: September 11, 2023; Preliminary interim results are presented from the ongoing clinical studies.

Increasing Phenotypic Correction (MMC-resistance) over 1 to 3 Years Post RP-L102 in Pivotal Phase 2 Trial

BM MMC-resistance ≥20% at 12m in 7 of 12 patients
Sustained BM MMC-resistance confirmed in 6 patients *



7 of 12 patients had MMC-resistance of ≥20% at 12 months

For 6 patients, increased MMC-resistance in BM CFU (40% to 94%) was observed 18 to 24 months post RP-L102 *(confirmatory assessment pending for patient 12 (2024))*

BM, bone marrow; CFU, colony-forming units; MMC, mitomycin-C.
*One additional patient (Patient 1: 1001) was noted to have BM MMC resistance of 49% at ~40 months post–RP-L102 infusion (Unscheduled visit, not shown) and ~41% at 48 months post–RP-L102 infusion.
Data cut-off: September 11, 2023; Preliminary interim results are presented from the ongoing clinical studies.

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Development Plan

Moving toward BLA/MAA submission

INITIAL EFFICACY AND HIGHLY FAVORABLE SAFETY PROFILE

- 7 of 11 patients evaluable for efficacy are clinical therapeutic successes based on >=18 months of data.
- No cytotoxic conditioning, only 1 transient RP-L102 related SAE (Grade 2)

TOP-LINE DATA READOUT ACHIEVED

- Met clinical criterion of at least 5 patients achieving primary composite endpoint, including BM MMC resistance at least 20% at 12 months and confirmed at 18- or 21-months post-infusion.

NEXT STEPS

- **MAA submission accepted – under review**
- **Anticipated BLA acceptance in 2024**

Additional life-cycle management activities:

- Expansion to FANC C and G
- Exploration of non-genotoxic conditioning and HSC expansion

REGULATORY DESIGNATIONS:

- RMAT, PRIME
- Orphan Drug designation in the US/EU
- Rare Pediatric Disease designation (eligible for PRV)
- Fast Track (US), ATMP

ATMP, advanced therapy medicinal product; BLA, Biologics License Application; CMC, chemistry, manufacturing, and controls; FANC, Fanconi Anemia complementation group; FDA, Food and Drug Administration; HSC, hematopoietic stem cell; MAA, Market Authorization Application; MMC, mitomycin-C; PRIME, Priority Medicines; PRV, priority review voucher; RMAT, Regenerative Medicine Advanced Therapy; SAE, severe adverse event. SAE observed in Rocket-sponsored study. Data cut-off: September 11, 2023.

RP-L201 for LAD-I: *ITGB2* Gene Mutation

Market Opportunity – US and EU
Prevalence of **800 to 1,000** individuals
Annual incidence of **50 to 75** individuals

Disease etiology

- ***ITGB2* gene mutations (21q22.3), encoding the beta-2-integrin, CD18; essential for leukocyte adhesion to endothelium**
- **CD18 absent or reduced on neutrophils**

Therapeutic challenges

Standard of care:
- Allogeneic HSCT

Limitations:
- Donor availability
- Infections
- Frequent GvHD
- Graft failure

Clinical manifestations

Patients suffer from recurrent infections; fatal in majority
- Severe LAD-I: Death prior to age 2 in 60% to 75% of patients, infrequent survival >5 years in absence of allogeneic HSCT
- Moderate LAD-I: Death prior to age 40 in >50% of patients, extensive morbidity with recurrent infections and inflammatory lesions

CD18, cluster of differentiation 18; GvHD, graft-versus-host disease; HSCT, hematopoietic stem cell transplant; *ITGB2*, integrin subunit beta 2; LAD-I, leukocyte adhesion deficiency-I.
Almarza NE, Kasbekar S, Thrasher AJ, et al. Leukocyte adhesion deficiency-I: A comprehensive review of all published cases. J Allergy Clin Immunol Pract. 2018;6(4):1418-1420.e10.

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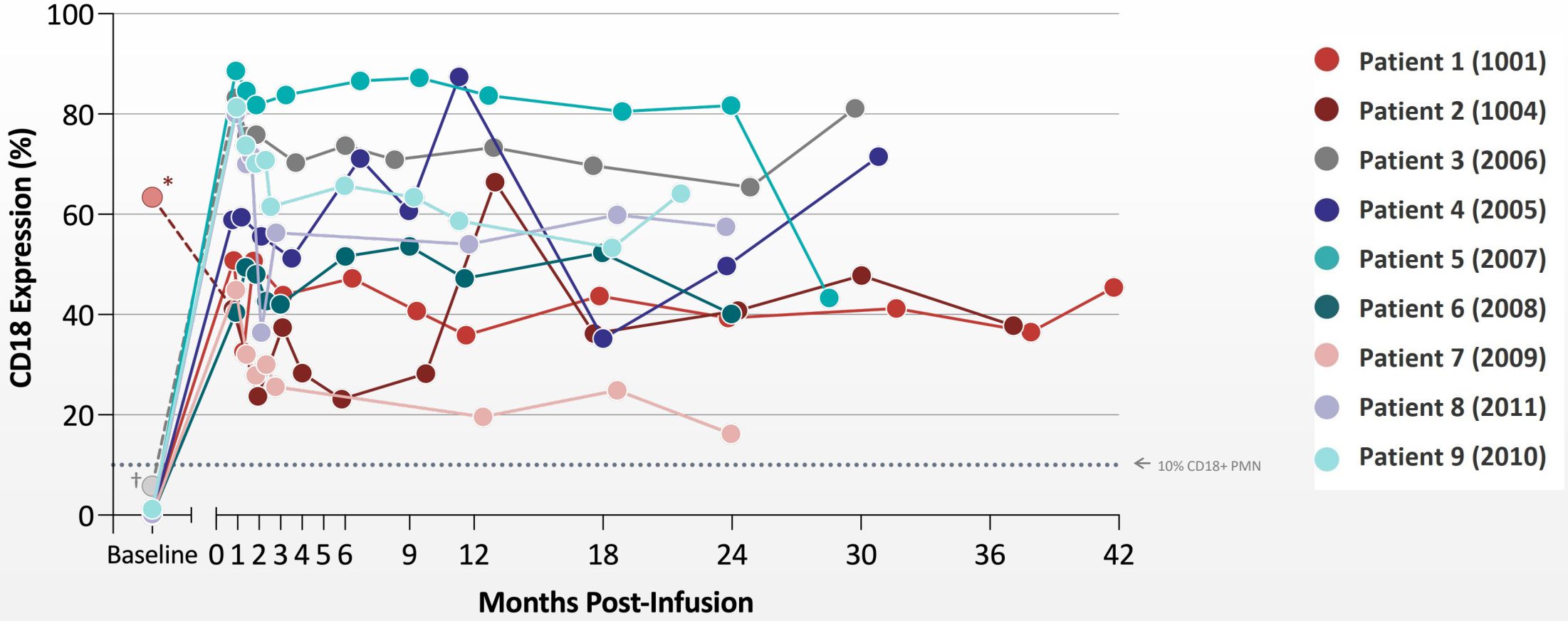
CD18 Expression in PB Polymorphonuclear Cells (PMNs) in Pivotal Phase 1/2 Trial

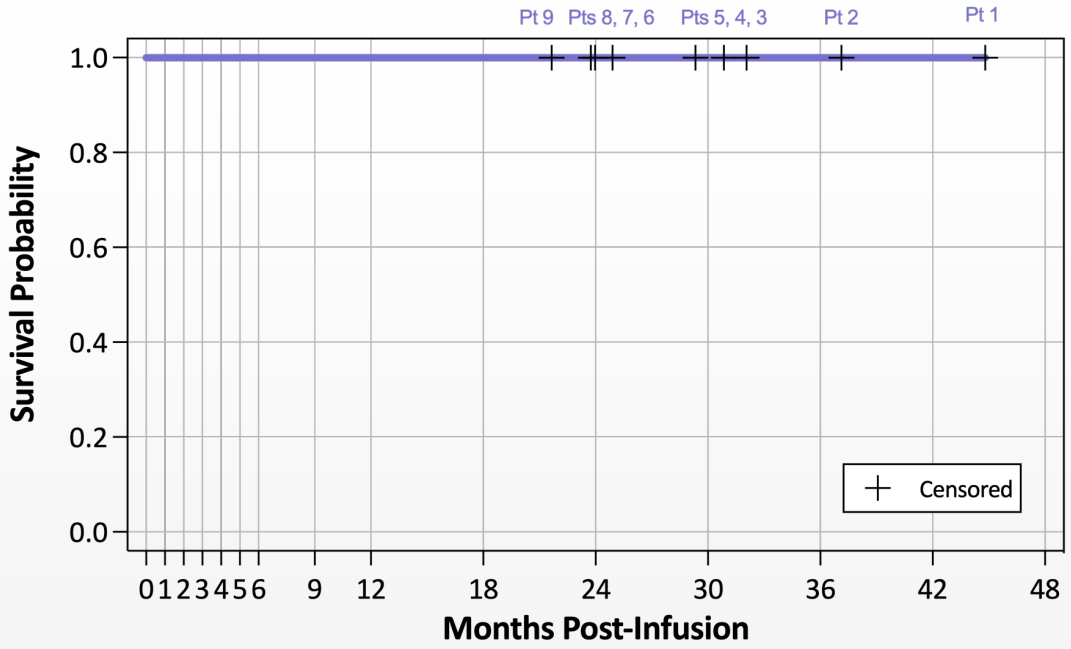
Sustained >10% PMN CD18 expression 1 year after gene-corrected cell infusion across the entire cohort



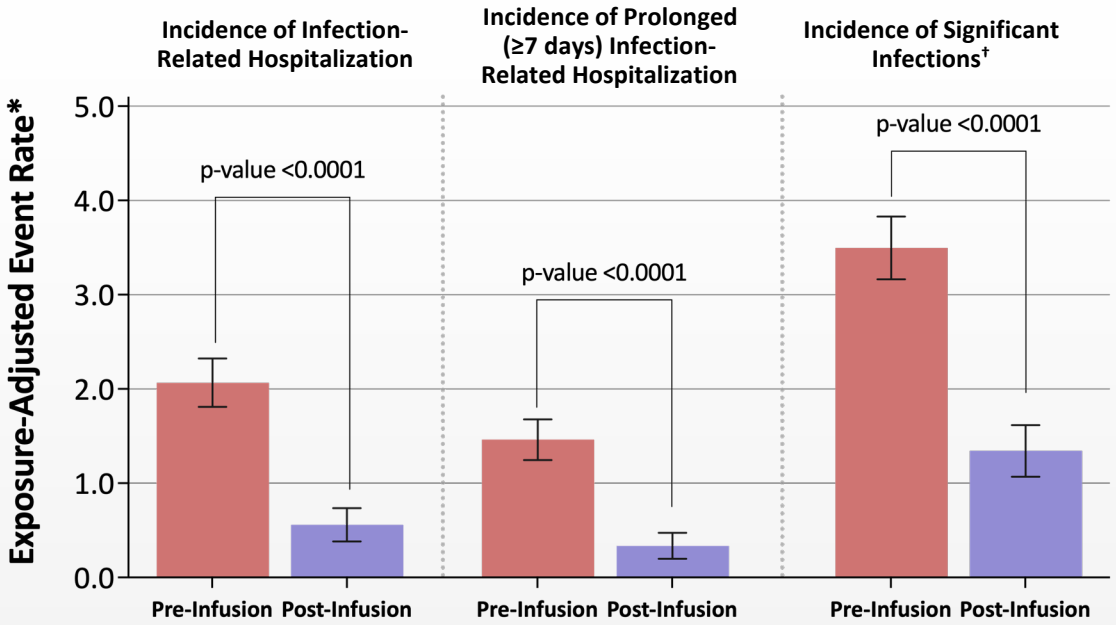
Neutrophil CD18 expression is reported utilizing CD18 monoclonal antibody (clone 6.7).
* Dim/weak CD18 expression reported at baseline for Patient 2 (1004) in ~63% of cells in conjunction with <2% CD11a/CD11b expression, likely indicating abnormal/unstable protein.
† Dim/weak CD18 expression reported at baseline for Patient 3 (2006) in ~5.8% of cells in conjunction with <2% CD11a/CD11b expression, likely indicating abnormal/unstable protein.
LAD-I, Leukocyte Adhesion Deficiency-I; PB, peripheral blood; PMN, polymorphonuclear neutrophil.
Data on file. Rocket Pharmaceuticals. 2024. Data Cut-Off: July 24, 2023. RP-L201-0318 120-Day Efficacy Update.
Pt 5 (2007) VCN at 30m timepoint remained stable relative to prior months, consistent with aberrant (artifactually low) CD18 result

Significant Reduction in Hospitalizations and 100% HSCT-free Survival in Pivotal Phase 1/2 Trial

100% HSCT-free survival Kaplan–Meier estimate



Meaningful reduction in infection-related hospitalizations following immune reconstitution



Survival without allogeneic HSCT

Primary outcomes

- ≥1-year post–RP-L201 infusion AND
- ≥2 years of age for subjects enrolled <1 year of age

- ***Infections that developed beyond 90 days post-infusion were consistent with typical childhood infections*** *frequently observed in immunocompetent (healthy) children*
- ***All patients have been able to stop prophylactic antibiotics*** *(when permitted by institutional policy)*

* Annualized event rate is calculated as the Total Number of Events / Total Time in each Time Period. Results are adjusted ev ent rate per year. Pre-infusion includes all lifelong medical history prior to RP-L201 infusion. p-values from Poisson regression with event and time period in the model with an offset of log exposure.
† Significant infections are defined as those requiring hospitalization or I.V. antimicrobial therapy.
HSCT, hematopoietic stem cell transplantation; I.V., intravenous; LAD-I, leukocyte adhesion deficiency-I.
Data on file. Rocket Pharmaceuticals 2024. Data Cut-Off: July 24, 2023; RP-L201-0318 120-Day Efficacy Update.

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Development Plan

Moving toward product filing

ENROLLMENT AND INITIAL EFFICACY

- Enrollment completed; 9/9 patients treated
- Efficacy observed in 9/9 patients with 12 to 36 months follow-up
- Efficacy is comprehensive, across all efficacy parameters including CD18 expression and survival

TOP-LINE DATA READOUT Q2 2022

- Survival for 9/9 patients, ≥2 years age and ≥1 year post-treatment
- No graft failure, GvHD
- No RP-L201 related SAEs

NEXT STEPS

- **FDA Approval**
- **Establish therapy as a safe and effective treatment option for LAD-I patients**
- **Create a commercial infrastructure that can be leveraged for future programs and franchises**

Life-cycle management

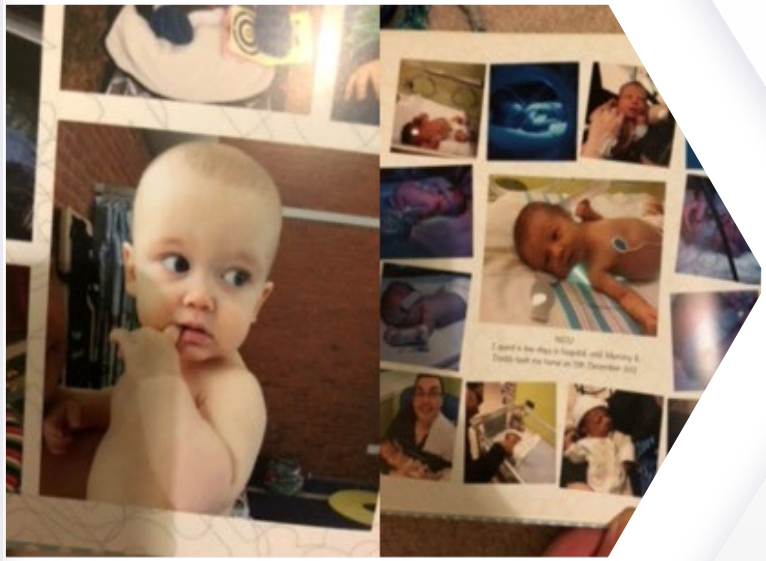
- Potential label expansion to include moderate LAD-I population

REGULATORY DESIGNATIONS:

- RMAT, PRIME
- Orphan Drug designation in the US/EU
- Rare Pediatric Disease designation (eligible for PRV)
- Fast Track (US), ATMP

FDA, Food and Drug Administration; NHS, National Health Service; PKD, pyruvate kinase deficiency; RMAT, Regenerative Medicine Advanced, PRIME, Priority Medicines, PRV, priority review voucher.
Data on file. Rocket Pharmaceuticals. 2024.

RP-L301 for PKD: ***PKLR*** Gene Mutation



Market Opportunity – US and EU
Prevalence of **4,000 to 8,000** individuals
Annual incidence of **75 to 125** individuals

Disease etiology

- Autosomal recessive inheritance
- Pyruvate kinase deficient RBCs cannot synthesize ATP, resulting in hemolytic anemia

Therapeutic challenges

- Standard of care: Chronic blood transfusions and splenectomy
- Limitations:
  - Iron overload
  - Extensive end-organ damage
  - Splenectomy confers lifelong infection and thrombotic risk

Clinical manifestations

- Lifelong chronic hemolysis
- Other clinical manifestations:
  - Anemia
  - Jaundice
  - Iron overload

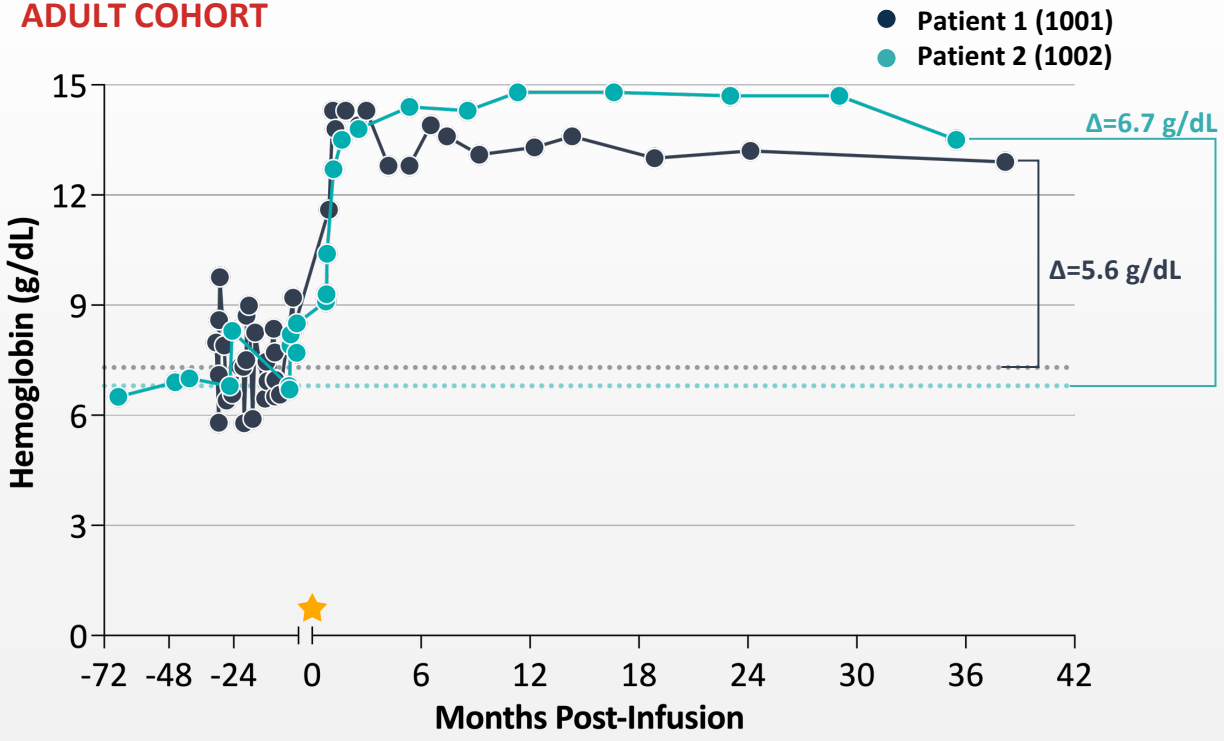
ATP, adenosine triphosphate; PKD, pyruvate kinase deficiency; PKLR, pyruvate kinase L/R; RBC, red blood cell.

Zanella A, Fermo E, Bianchi P, Valentini G. Red cell pyruvate kinase deficiency: molecular and clinical aspects. Br J Haematol. 2005;130(1):11-25.

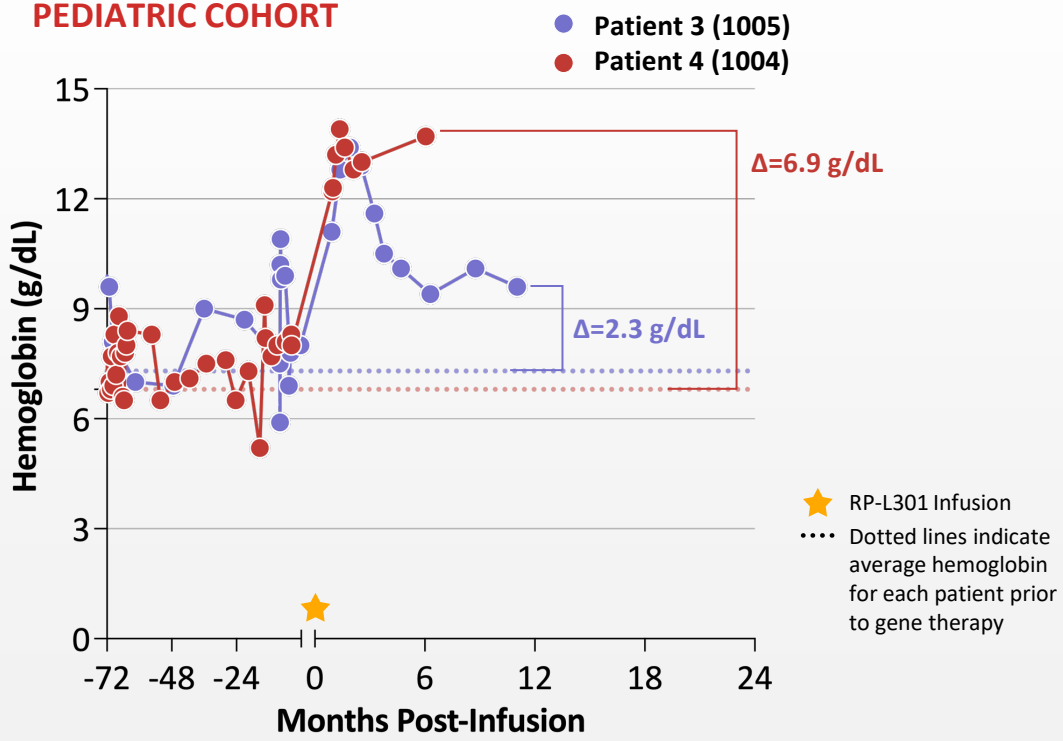
Preliminary Phase 1 Efficacy Results: Adult and Pediatric Patients

Sustained & meaningful hemoglobin improvements from severe (<8 g/dL) baselines
No RBC transfusions required following neutrophil engraftment
Concurrent improvement across biochemical markers

ADULT COHORT



PEDIATRIC COHORT



The average baseline Hb is determined by Hb values from 2y prior to enrollment to immediately prior to stem cell mobilization , excluding those impacted by RBC tx. Post-transfusion Hb values within 61d of a prior RBC tx were excluded unless the reported Hbb value was a pre-tx assessment for a subsequent RBC tx within 3 days of the prior RBC tx date.
Hb, hemoglobin; RBC, red blood cell; Tx, transfusion
Data on file. Rocket Pharmaceuticals. 2024. Data cut-off: February 5, 2024; preliminary interim results are presented from the ongoing clinical study.

Development Plan

Alignment reached with FDA on pivotal Phase 2 trial design

PLAN FOR PHASE 2 AND LAUNCH

High level pivotal Phase 2 Trial Design

- Single-arm, 10 patient study
- Primary endpoint of ≥1.5-point Hgb improvement at 12 months
- Supports accelerated approval

Well-delineated natural history in recent PKD NHS publications

REGULATORY DESIGNATIONS

Fast Track, RMAT, Orphan Drug (US/EU), PRIME, Rare Pediatric Disease (eligible for PRV)

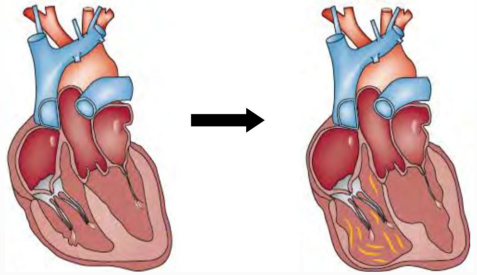
NEXT STEPS

- **Initiation of Pivotal Phase 2 Study**

FDA, Food and Drug Administration; NHS, National Health Service; PKD, pyruvate kinase deficiency; RMAT, Regenerative Medicine Advanced, PRIME, Priority Medicines, PRV, priority review voucher.
Data on file. Rocket Pharmaceuticals. 2024.

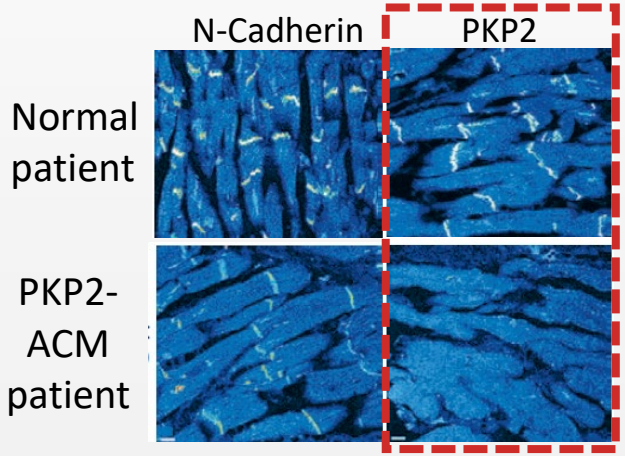
PKP2-Arrhythmogenic Cardiomyopathy (ACM)*:
A high-risk disease with no curative options

Advanced ACM Heart with fibrofatty replacement in right ventricle



Electrical manifestations can precede structural abnormalities

ACM: Diminished Myocardial PKP2



Disease Etiology

- Autosomal dominant mutations in *PKP2* gene, which encodes for Plakophilin-2, a component of the desmosome localized to cardiac intercalated discs

Therapeutic Challenges

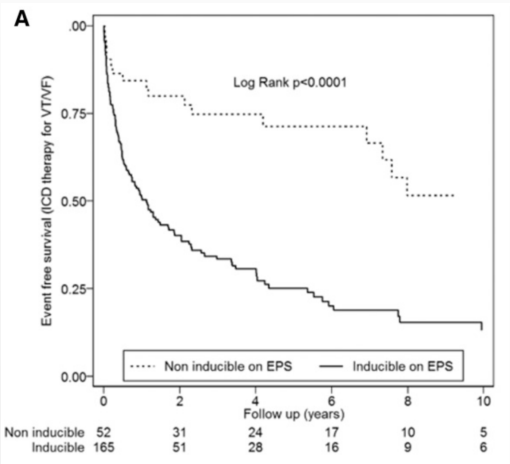
- Current standard of care includes beta-blockers, anti-arrhythmic agents, and ablation
- Available treatments do not modify disease progression; no curative therapeutic options

Clinical Manifestations

- Mean age at presentation: 35y (±18) [1]
- 5-10% annual risk of sustained ventricular arrhythmias (VA), with higher risk in patients who present with symptoms of disease (index patients)[2-3]
- In one study, >70% risk of VAs in index patients (median follow up, 7 years) [4]
- ICD placement in >80% of index patients [5]
- For patients with ICDs:
  - 45-75% will have ICD firing (shock) over 3-5 years
  - ≥50% 2-year incidence of firing in subgroups:
    - male; • EPS-induced VT; • history of VT;
    - ≥3 ECG leads with TWI; • >1000 PVC/24h [5-6]

Estimated Prevalence (US+EU): ~50,000

Kaplan-Meier Incidence of ICD Firing



Event free survival in ACM patients who underwent EP study prior to placement of an ICD

- *~70% of patients who were inducible on EP study had an ICD firing at 2 years*

Biopsy figure adapted from: Asimaki et al. NEJM, 2009; Table adapted from Dalal et al. Circulation 2006. SOC: standard of care; CM: cardiomyopathy; HF: heart failure: HTx: heart transplantation; RV: Right ventricular; SD: Standard Deviation; VT: ventricular tachycardia; LBBB: left bundle branch block; ICD: implantable cardioverter defibrillator; RVEF: right ventricular ejection fraction; LV: left ventricle; SVA: sustained ventricular arrhythmia.

*This cardiomyopathy initially manifests in the right ventricular free wall, so the disease was termed arrhythmogenic right ventricular dysplasia/cardiomyopathy (ARVD/ARVC). However, since left dominant and biventricular forms have also been observed, this has led more recently to the use of the term "ACM". 1. Bhonsale. EHJ 2015; 36: 847-55. 2. Towbin JA. Heart Rhythm 2019;16(11). 3. Cadrin-Tourigny J. Eur Heart J 2022;43. 4. Groenweg. Circ Cardiovasc Genet 2015; 8: 437-46. 5. Calkins. Circ 2017; 136: 2068-82. 6. Orgeron. J Am Heart Assoc 2017: e006242.

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PKP2-ACM Prevalence in the US and EU

ACM prevalence

1:1000 to 1:5000

Peters 2004, McKenna 2021

PKP2 variants

32.9%

2,572 ACM patients assessed from 13 publications an aggregated mean of **32.9% had *PKP2* mutations**[1]

ACM-PKP2 US & EU Prevalence

~50,000

Utilizing the conservative ACM prevalence (1:5000) and the 32.9% PKP2 mutation frequency in ACM

Peters S, Trümmel M, Meyners W. Prevalence of right ventricular dysplasia-cardiomyopathy in a non-referral hospital. Int J Cardiol. 2004;97(3):499-501.
McKenna WJ, Judge DP. Epidemiology of the inherited cardiomyopathies. Nat Rev Cardiol. 2021;18(1):22-36.
[1] Data on file

Proof of Concept in Translationally Relevant Animal Model

Completed RCKT Studies with *Cardiomyocyte-specific PKP2 Knockout Mouse Model of ACM*

- Initial POC evaluated 4 AAV Vectors: Cardiac Functional & Structural Analyses
- Dose-related effects evaluated with 2 AAV vectors: Cardiac Functional & Structural Analyses
- Evaluated Survival, Functional, and Anatomic Benefit in 'Arrest Progression' Models
  - Including delivery of AAV *+7 or +14 Days after* induction of PKP2 knockout and subsequent disease onset

Analyses Include:

- Survival
- Echocardiography and ECG
- PKP2 expression (IF and WB)
- Cardiac pathology & fibrosis
- Vector DNA, transgene mRNA
- General safety including pathology

Completed sponsored research.

Academic Partner:

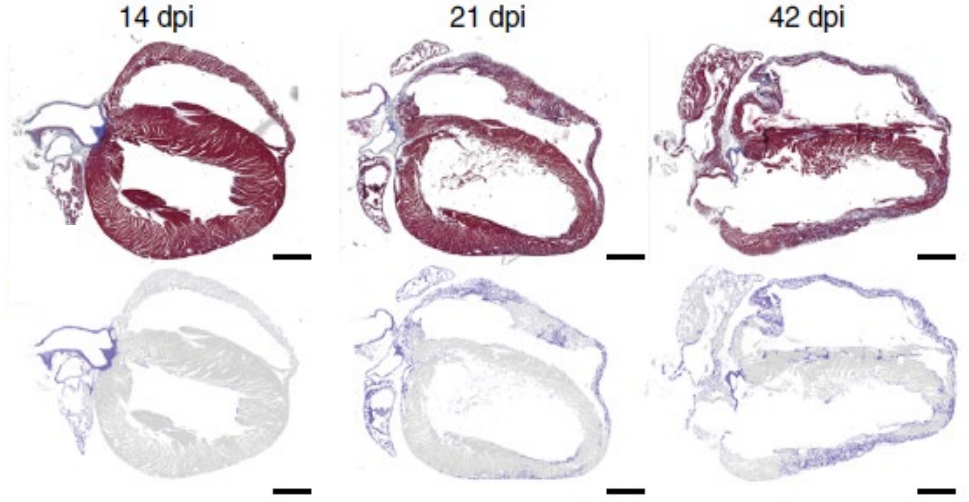
NYU Grossman School of Medicine

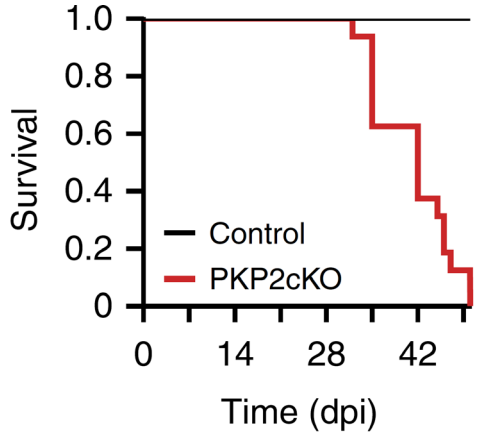
Mario Delmar, MD, PhD
Patricia and Robert Martinsen Professor of Cardiology,
Department of Medicine; Division of Cardiology,
NYU Grossman School of Medicine

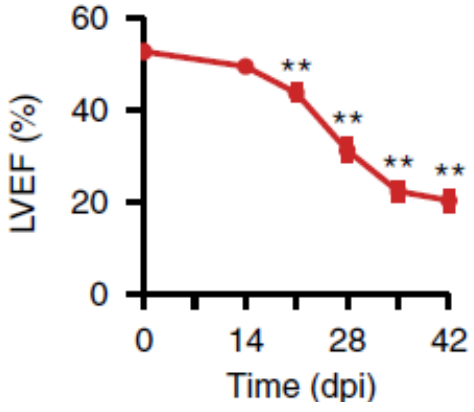
Marina Cerrone, MD
Research Associate Professor,
Co-Director, Inherited Arrhythmia Clinic,
Department of Medicine; Division of Cardiology,
NYU Grossman School of Medicine

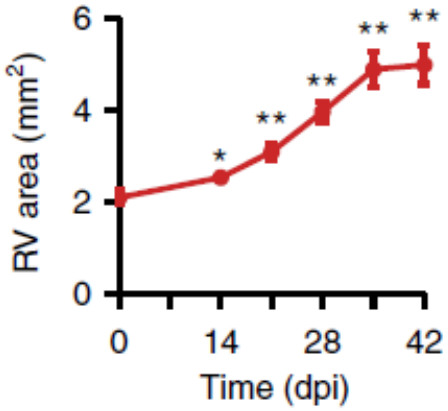
Tamoxifen-induced ACM in the PKP2-cKO Mouse Model

- The PKP2-cKO mouse model recapitulates ACM following induction of PKP2 KO by tamoxifen (TAM) injection
- Progression of cardiomyopathy evidenced by Masson's trichrome staining of heart sections in PKP2-cKO mice from 14 to 42 days post-TAM (dpi)
- 100% mortality by day ~50 following TAM injection
- Left ventricular ejection fraction (LVEF) diminishes significantly across time
- Right ventricular (RV) enlargement occurs across time
- Premature Ventricular Contractions (PVCs) are a clinical hallmark of ACM and emerge in the animal model because of Pkp2 loss









Cerrone M, Montnach J, Lin X, et al. Plakophilin-2 is required for transcription of genes that control calcium cycling and cardiac rhythm. Nat Commun. 2017;8(1):106. Published 2017 Jul 24.
ISO, isoproterenol; cKO: conditional knockout

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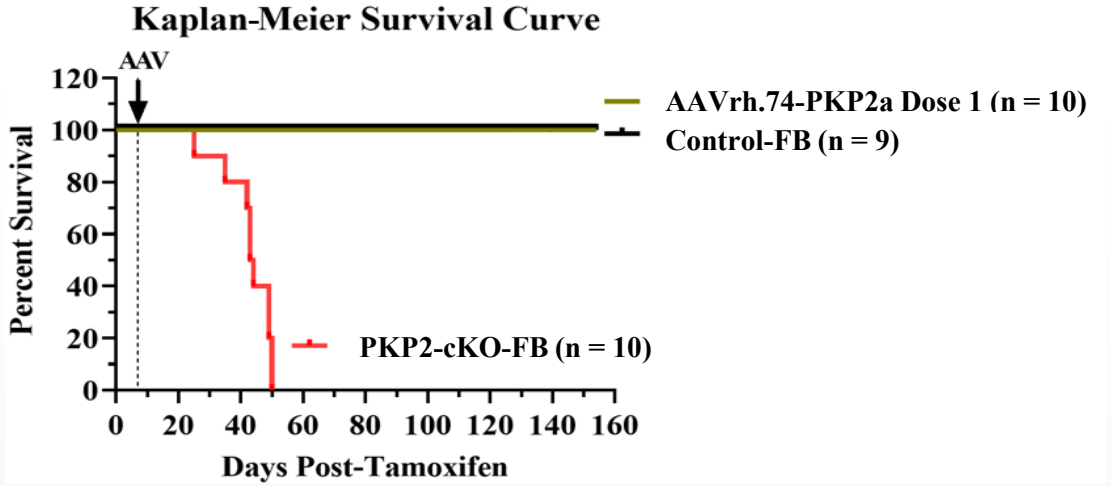
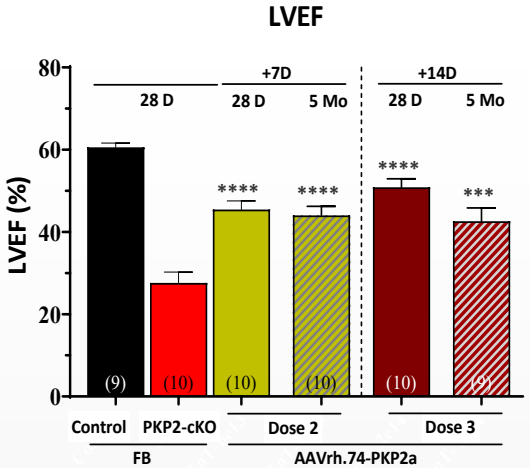
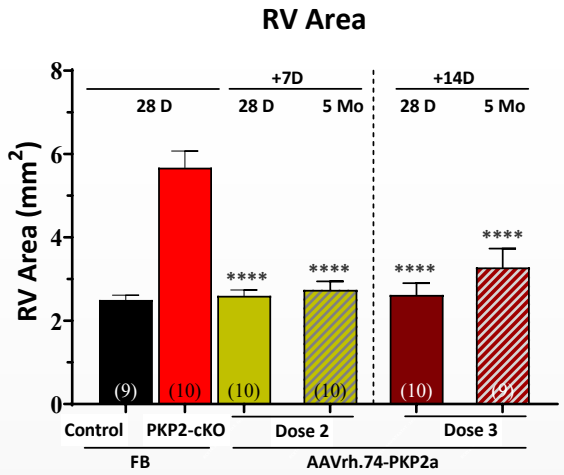
Increased Survival & Preserved Cardiac Function in the PKP2-cKO Model

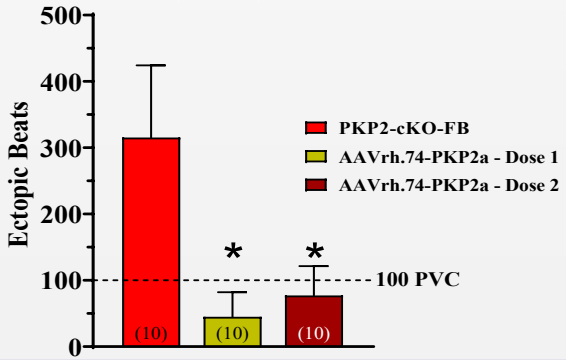


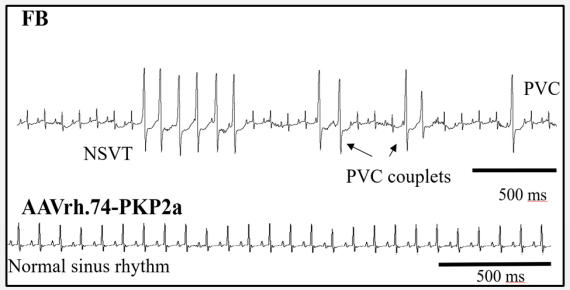




- AAVrh.74-PKP2 delivered ***7 days post-TAM***:
  - **100% survival to 5 months,** compared to 100% mortality by day ~50 in PKP2-cKO control animals
  - **Preserved Ejection Fraction and Right Ventricular Area** at 28 Days, sustained to 5 months
- AAVrh.74-PKP2 delivered ***14 days post-TAM:***
  - Mitigated isoproterenol-induced PVCs and arrhythmia, disease-related characteristics of ACM
  - Robust benefit on survival, cardiac function & structure to 5 months[1]

ISO-Induced Arrhythmia
AAV+14D Post-TAM; ECG at 21D post-TAM





*p <0.05 vs PKP2-cKO FB
ISO = isoproterenol; TAM = tamoxifen; ECG = Electrocardiography

[1]Data not illustrated in Figures

Optimal Gene Therapy for PKP2-ACM Expected to be First-and Best-In-Class

cDNA/isoform:

- **PKP2a:** full wild type coding sequence of therapeutic gene, protein loss drives ACM

AAV Serotype:

- ***AAV.rh74* serotype associated with favorable safety profile in DMD/LGMD2E[1-3]; potential for safe administration at optimal doses for adult ACM patients**

Cardiac-Specific Promoter:

- Effectively drives expression of therapeutic transgene in cardiomyocytes; minimizes off-target effects

Route of Administration:

- **Intravenous (IV)** Pharmacology studies demonstrate efficient cardiac transduction with IV administration

Robust Proof of Concept in Disease Relevant Animal Model:

- **NYU Cardiac-specific cKO-PKP2 mouse** (biologically relevant translational model)

[1]Rodino-Klapac et. al. Safety, β-Sarcoglycan Expression, and Functional Outcomes From Systemic Gene Transfer of rAAVrh74.MHCK7.hSGCB in LGMD2E/R4. Presented at the Muscular Dystrophy Association (MDA) Conference. Nashville, TN, March 13–16, 2022.
[2]Mendell et. al. A Phase 2 clinical trial evaluating the safety and efficacy of delandistrogene moxeparvovec (SRP-9001) in patients with Duchenne muscular dystrophy. Presented at the 2022 Muscular Dystrophy Association (MDA) Conference Nashville, TN, March 13–16, 2022.
[3] van Opbergen et al., Circ Genom Precis Med, 2024; Feb;17(1):e004305. DOI: 10.1161/CIRCGEN.123.004305.

Clinical Development Plan

Phase 1 Dose Escalation Study

Completed or Ongoing Activities

- ✓ **Phase 1 Study Initiated**
- ✓ **Orphan Disease Designation**
- ✓ GMP drug product manufacturing completed
- ✓ Pharmacology and GLP toxicology studies
- ✓ Potency assay
- ✓ Clinical protocol developed, vetted by Scientific Advisory Board and informed by patient insights
- ✓ Launching multi-center, clinical trial

High Level Phase 1 Trial Design

- Study design:
  - FIH, multi-center, dose escalation study to assess safety and preliminary efficacy
  - Starting dose of 8 x 10^{13} GC/kg
  - Target population: Adult PKP2-ACM patients with ICDs and high risk for arrhythmias
- Primary endpoint:
  - Safety events
- Secondary and exploratory endpoints:
  - PKP2 tissue protein expression
  - Clinical markers of life-threatening ventricular arrhythmias
  - Cardiac biomarkers

Natural History

- Natural history studies are planned to provide context for the Phase 1 trial and additional information on the progression of PKP2-ACM

FDA, Food and Drug Administration; FIH, first in human; ICD, implantable cardioverter defibrillators; PKP2: plakophilin 2; ACM: Arrhythmogenic Cardiomyopathy

BAG3 Regulates Critical Functions in Cardiomyocytes

Cardiac contractility

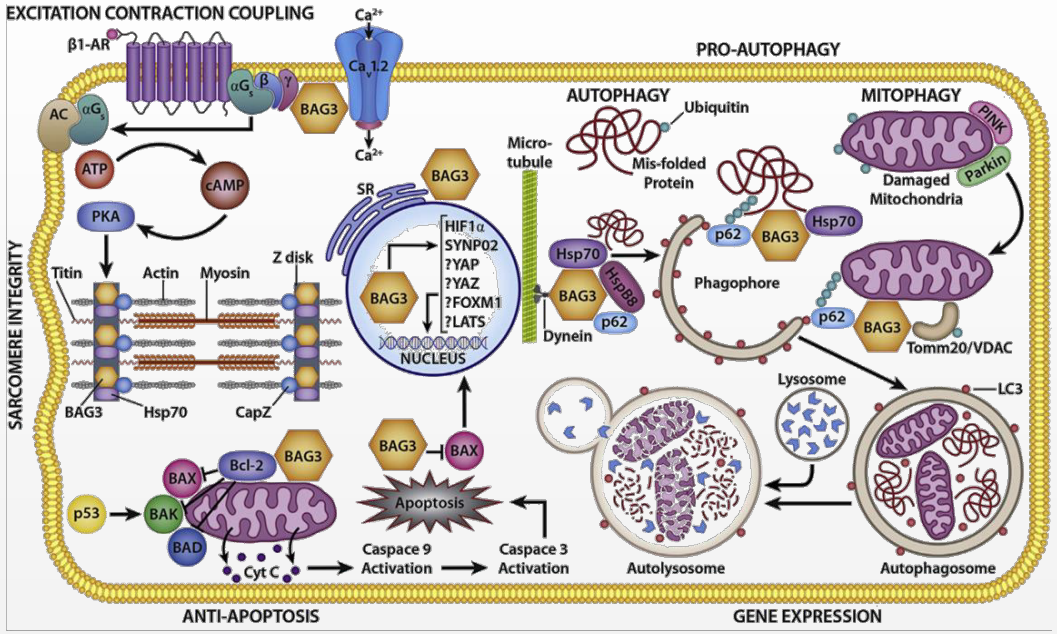
1

Enhances contractility by linking the β-adrenergic receptor and L-type Ca^{2+} channel

Structural support

2

Provides support for the sarcomere by linking actin myofibrils with the Z-disc



3

Protein quality control

Facilitates autophagy as a co-chaperone with heat shock proteins, recycling misfolded proteins

4

Anti-apoptosis

Inhibits apoptosis (programmed cell death) through binding of BCL2

We believe that a gene therapy approach is best positioned to restore the broad biological functions of BAG3 in the heart

BAG3, BLC2-associated athanogene 3; BCL2, B-cell lymphoma 2.

Knezevic T, Myers VD, Su F, et al. Adeno-associated Virus Serotype 9 - Driven Expression of BAG3 Improves Left Ventricular Function in Murine Hearts with Left Ventricular Dysfunction Secondary to a Myocardial Infarction. JACC Basic Transl Sci. 2016;1(7):647-656.
Myers VD, Gerhard GS, McNamara DM, et al. Association of Variants in BAG3 With Cardiomyopathy Outcomes in African American Individuals JAMA Cardiol. 2018;3(10):929-938.

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BAG3-DCM Opportunity and Next Steps

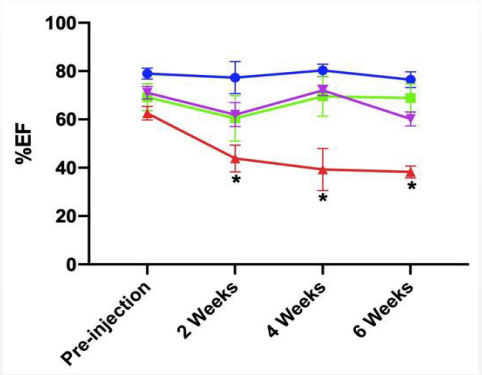
BAG3-DCM Represents a Significant Market with Unmet Need

- Dilated cardiomyopathy (DCM) is the most common form of cardiomyopathy
- 20% to 50% of DCM patients have familial DCM; up to 40% of whom have an identifiable genetic cause(1)
- Scientific societies recently endorsed clinical genetic testing for DCM patients and families(2,3)
- Prevalence of BAG3 DCM in US is estimated to be as high as 30,000 patients(4) and is expected to grow with increasing genetic testing and disease awareness

Initial Proof-of-Concept for AAV9-BAG3 Supports Further Development

- Initial proof of concept for AAV9-BAG3 demonstrated in BAG3-knockout mouse model

Ejection fraction in WT and BAG3 +/- mice treated at age 6 to 8 weeks with AAV9-GFP or AAV9-BAG3



WT mice: AAV-GFP or AAV-BAG3

BAG3 +/- mice: AAV9BAG3 treatment group

BAG3 +/- mice: Control group (AAV-GFP)

- Evaluating optimal development pathway
- IND planned for 2024

AAV, adeno-associated virus; BAG3, BCL2-associated athanogene 3; DCM, dilated cardiomyopathy; EF, ejection fraction; GFP, green fluorescent protein; IND, investigational new drug; WT, wild type. Haploinsufficiency data published in Myers VD et. al., *J Cell Physiol.* 2018; AAV-BAG3 administration adapted from data published in Myers VD et. Al., *JAMA Cardiol.* 2018; and unpublished data from the Feldman lab.

Cranbury R&D and Manufacturing Facility Overview

- Total Lab Space: ~30,000 sq. ft. for process development, analytical development, MS&T and QC
- Manufacturing capability from small-scale to toxicology-scale material
- Streamlined tech transfer timeline for pipeline assets from plasmid selection to IND in <15 months
- Manufacturing expansion to add media and buffer production capability
- Incorporating fully automated in-house vial filler suite
- Anticipated 2X capacity increase

Enables rapid, robust and cost-efficient internal development capability for new and existing programs in addition to full-scale commercial manufacturing

~100,000 ft^2

facility in Cranbury, NJ

MS&T, manufacturing, science and technology. QC, quality control. R&D, research and development. IND, Investigational new dru g.

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Future Therapies: Wave 2 (AAV)

Focused R&D Strategy for Sustainable Innovation

- **First-, best- and/or only-in-class**
- **On-target MOA; clear endpoints**
- **Sizeable market to maximize patient impact**
- **3 therapeutic areas (CV, heme and undisclosed)**

We continue to build our pipeline based on our core R&D strategy, identifying the "most impactful" indications for the most efficient development path.

AAV, adeno-associated virus; MOA, mechanism of action; R&D, research and development.

Expert Leadership With Proven Track Record

Gaurav Shah, M.D.
Chief Executive Officer
Spearheaded Kymriah (CART-19) development at Novartis towards approval

Kinnari Patel, Pharm.D., MBA
President, Head of R&D and Chief Operating Officer
Led Opdivo and six rare disease indication approvals

Jonathan Schwartz, M.D.
Chief Medical & Gene Therapy Officer
Led multiple biologics approvals

Aaron Ondrey
Chief Financial Officer
20+ years of experience in commercial finance, strategic planning, and M&A across multiple therapeutic areas

Mayo Pujols
Chief Technical Officer
~30 years technical operations and GMP manufacturing expertise

Mark White, MB.ChB.
General Manager, Commercial Affairs
Seasoned drug developer with 25+ years of industry experience

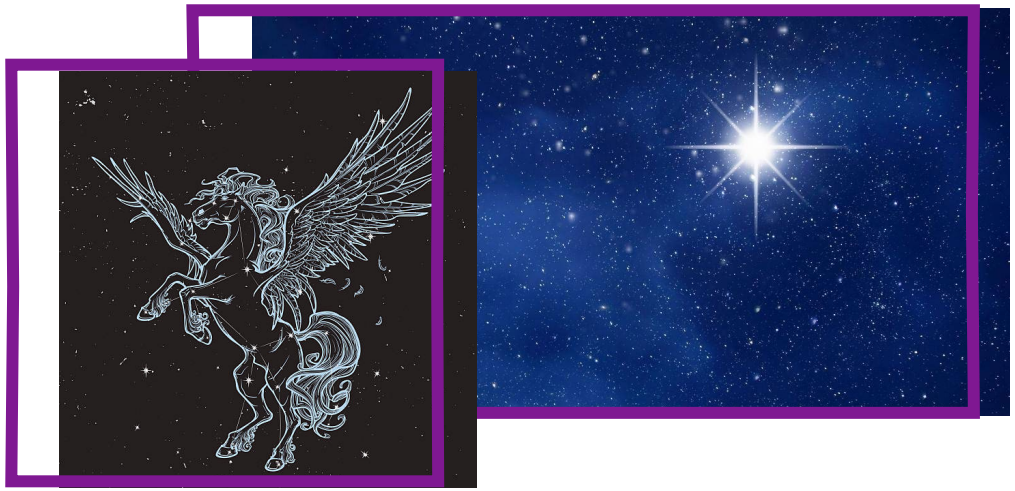
Martin Wilson, J.D.
General Counsel & Chief Corporate Officer
~20 years legal, compliance and executive experience and accomplishment in life sciences

Gayatri R. Rao, M.D., J.D.
Chief Regulatory Officer & SVP, Clinical Safety
7-year former Director of FDA's Office of Orphan Products Development

Raj Prabhakar, MBA
Chief Business Officer
~20 years cell, gene and biotech business development

Carlos Martin, BA, MBA
Chief Commercial Operations & Revenue Officer
15+ years global & local leadership, commercial strategy and new product launches

Isabel Carmona, J.D.
Chief People Officer
Seasoned leader in human resources, legal and compliance across life sciences, financial services and IT



THANK YOU!

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