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SEEKING GENE THERAPY CURES

DISCLAIMER

Various statements in this presentation concerning Rocket's future expectations, plans and prospects, including without limitation, Rocket's expectations regarding its guidance for 2023 in light of COVID-19, the safety, effectiveness and timing of product candidates that Rocket may develop to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD), Danon Disease (DD),PKP2-ACM (PKP2), BAG3-DCM (BAG3) and other diseases, and the safety, effectiveness and timing of related pre-clinical studies and clinical trials and related data readouts, may constitute forward-looking statements for the purposes of the safe harbor provisions under the Private Securities Litigation Reform Act of 1995 and other federal securities laws and are subject to substantial risks, uncertainties and assumptions. 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. 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 ability to monitor the impact of COVID-19 on its business operations and take steps to ensure the safety of patients, families and employees, the interest from patients and families for participation in each of Rocket's ongoing trials, our expectations regarding when clinical trial sites will resume normal business operations, our expectations regarding the delays and impact of COVID-19 on clinical sites, patient enrollment, trial timelines and data readouts, our expectations regarding our drug supply for our ongoing and anticipated trials, actions of regulatory agencies, which may affect the initiation, timing and progress of pre-clinical studies and clinical trials of its product candidates, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, and unexpected expenditures, 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, 2022, filed February 28, 2023 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.

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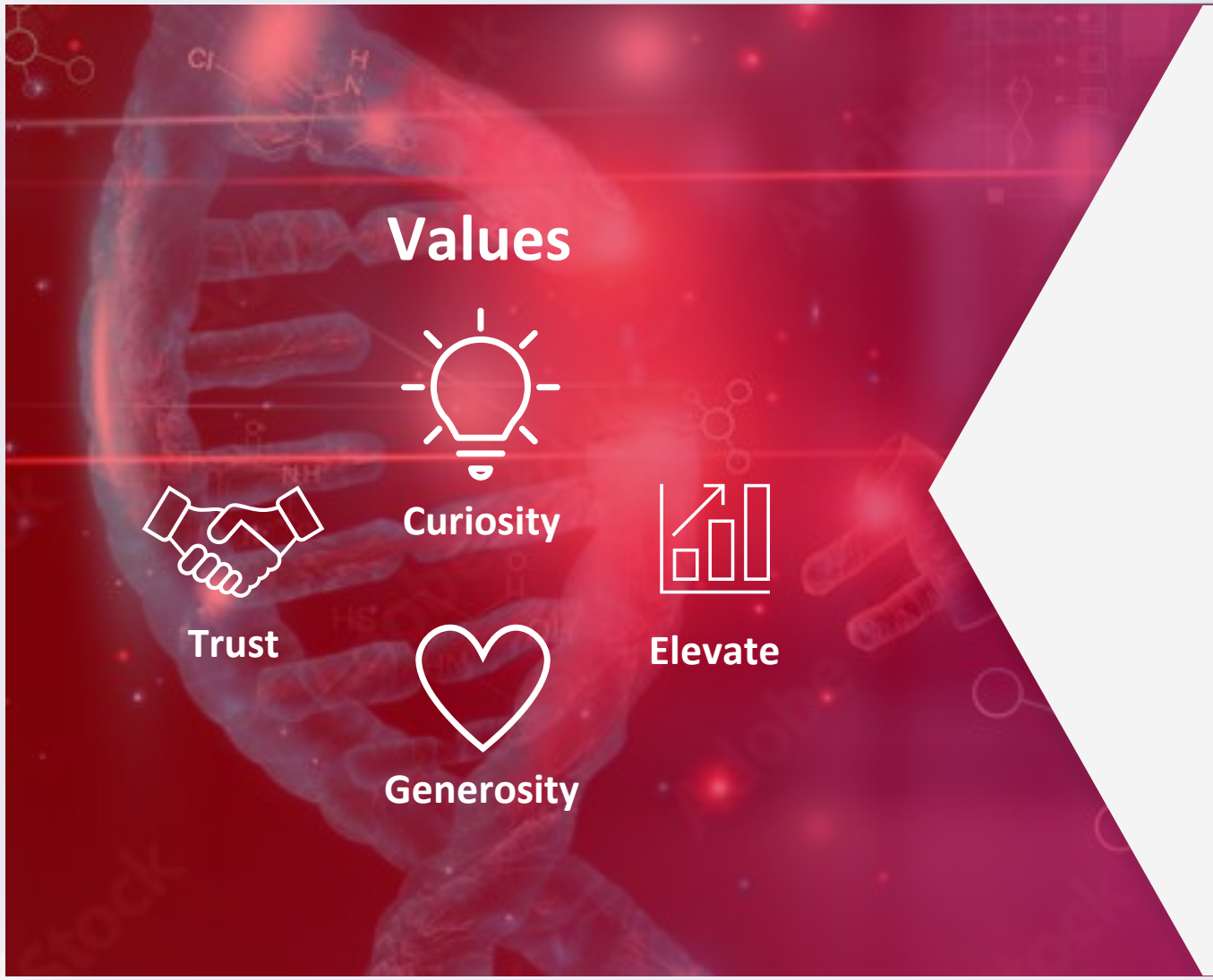
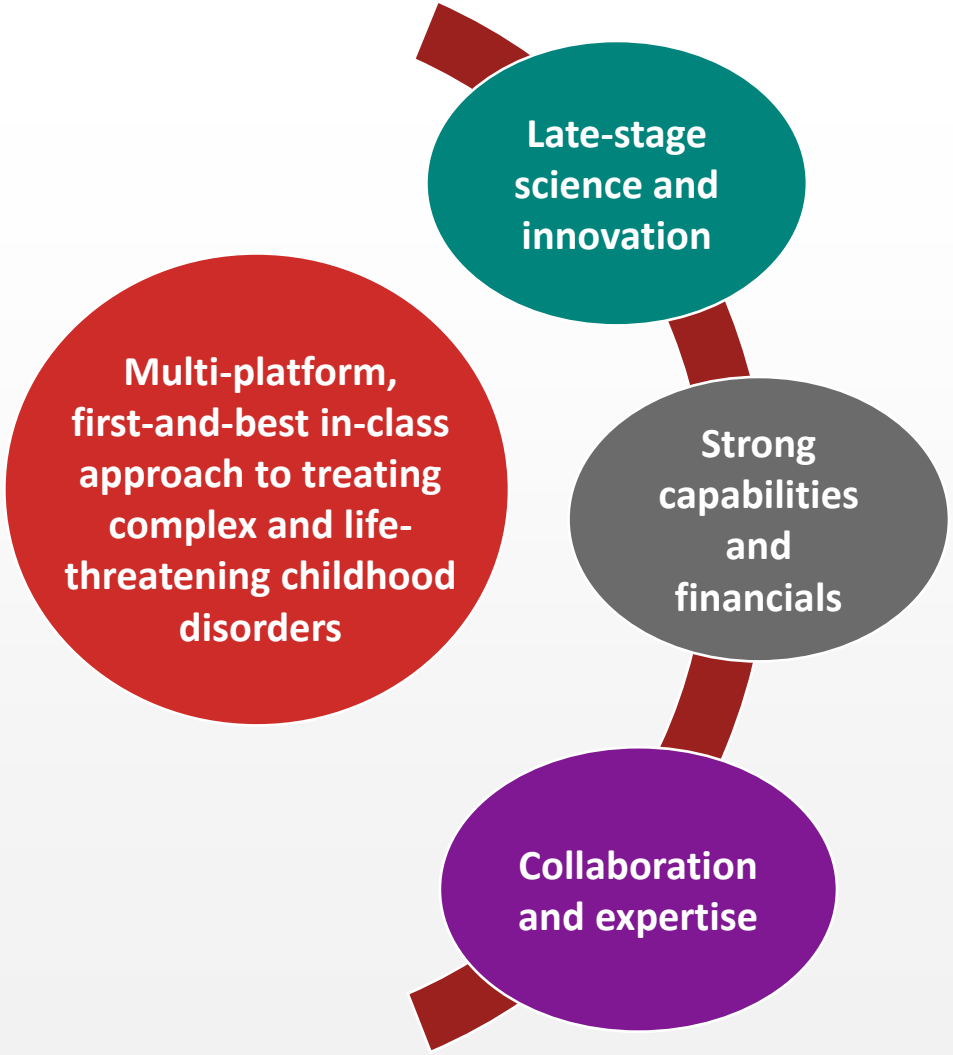
Vision: Seeking Gene Therapy Cures



Mission

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

Generating **Value-based** Gene Therapies



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

$307M[1]

in cash and cash equivalents; **sufficient to fund operations into 1H 2025**

Leadership team with proven track record of

20+

drug approvals and launches

World-class scientific experts and partners learning from and collaborating with **patient communities**

[1]Cash balance as of June 30, 2023.

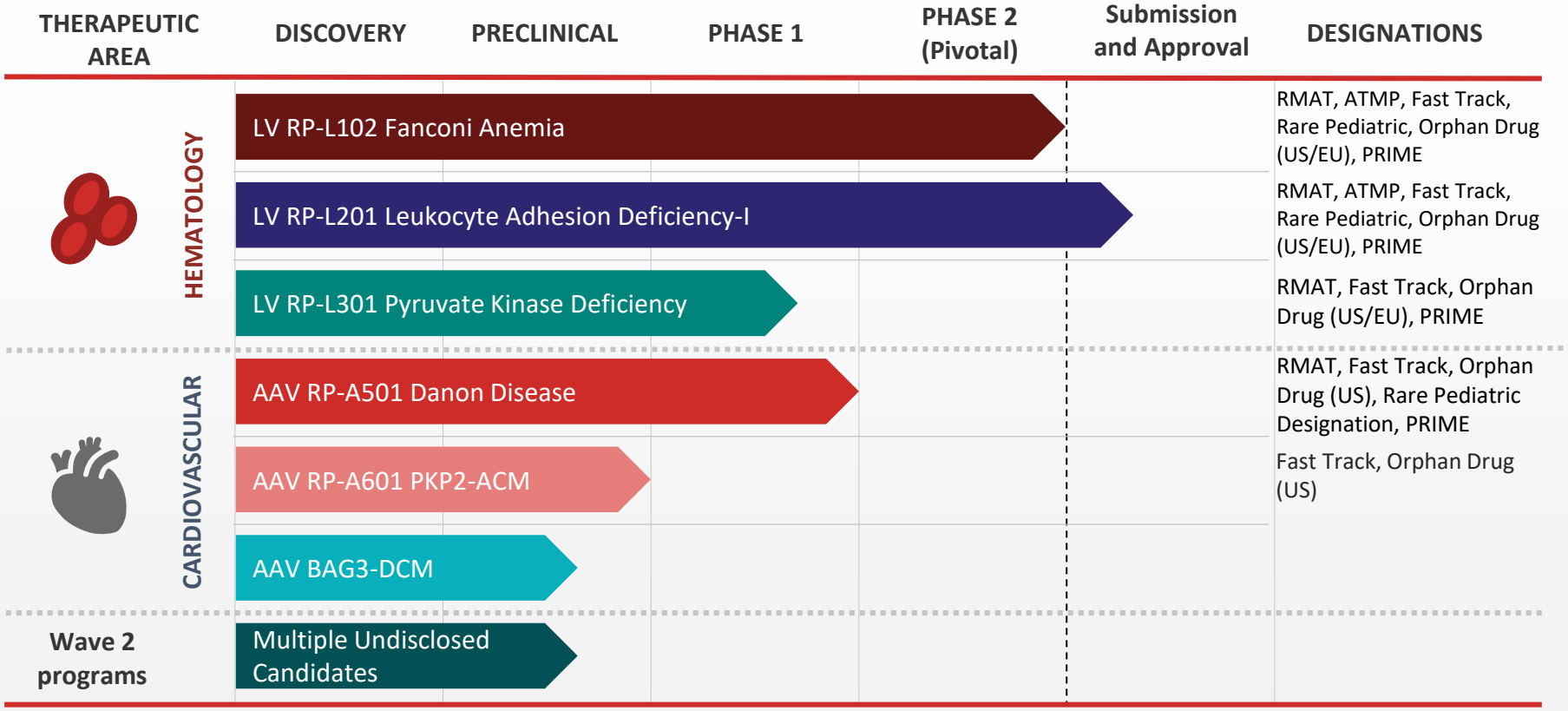
Strong Science, Carefully-selected Assets and Smart Execution:

6 Disclosed programs with compelling clinical and/or pre-clinical proof of concept

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
Data on file. Rocket Pharmaceuticals. 2023.

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

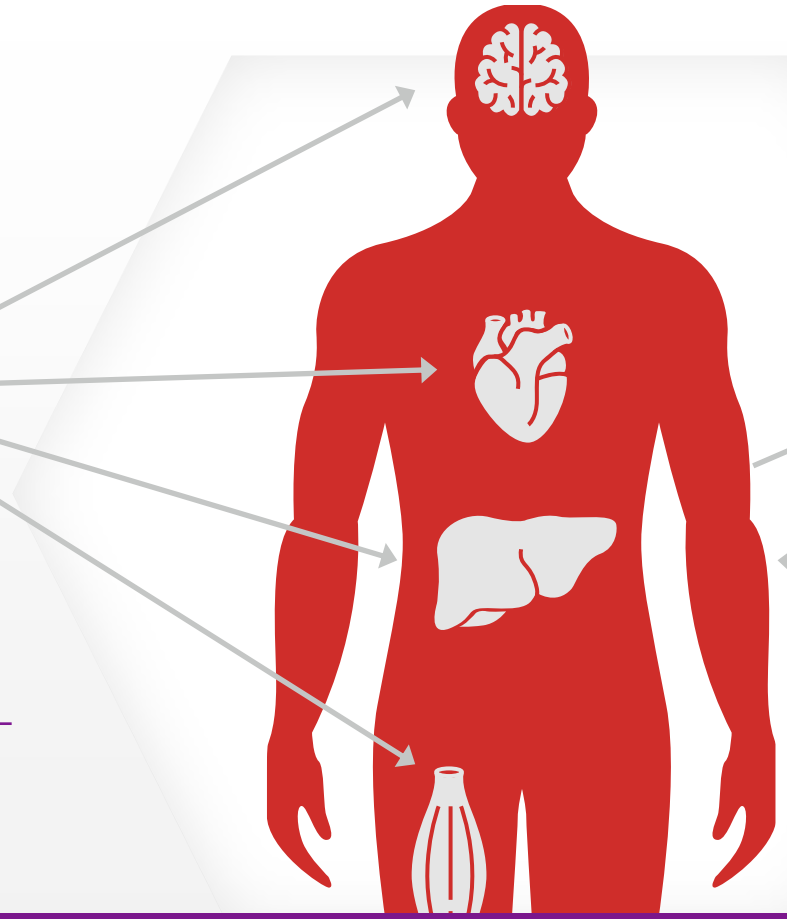
IN VIVO platform

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.
Data on file. Rocket Pharmaceuticals. 2023

Looking Forward to a Catalyst-Rich 2023

Transition from Clinical to Commercial Stage

2023

Q1

- Completed 2 In-house cGMP Danon Batches

Q2

- Planned Danon Phase 2 Study Initiation
- LAD-I Product Filing
- PKP2-ACM IND Filing Accepted by FDA

Q3

- Danon EU IMPD Filing

Q4

- FA Product Filing
- PKD Phase 2 Pivotal Study Initiation
- Danon Female Study Initiation
- LAD-I Moderate Study Initiation

2024

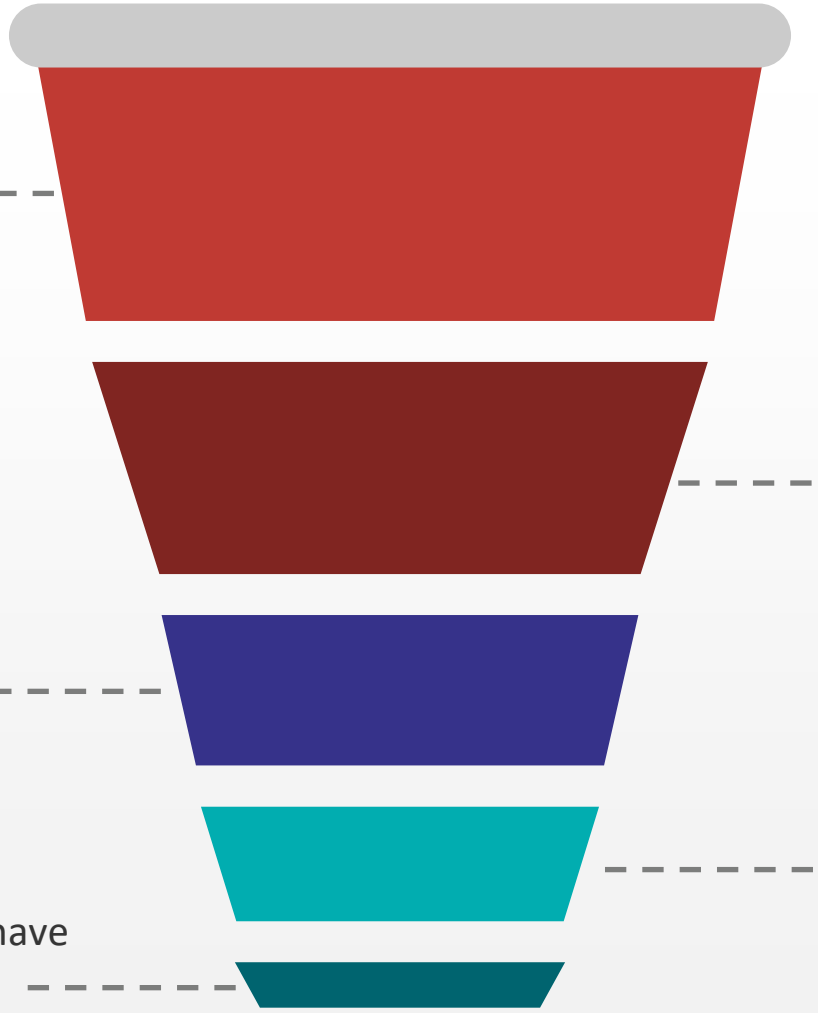
- BAG3 IND Filing
- FA (C & G) IND Submission
- Non-Genotoxic Conditioning for LV
- Additional Wave 2 Assets Disclosed

cGMP, current Good Manufacturing Processes; FA, Fanconi anemia; H1, first half of the year; 2H, second half of the year; LAD-I, leukocyte adhesion deficiency-I; PKD, pyruvate kinase deficiency; Q1, first quarter of the yearQ2, second quarter of the year; Q3, third quarter of the year; Q4, fourth quarter of the year.
Data on file. Rocket Pharmaceuticals. 2023.

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]

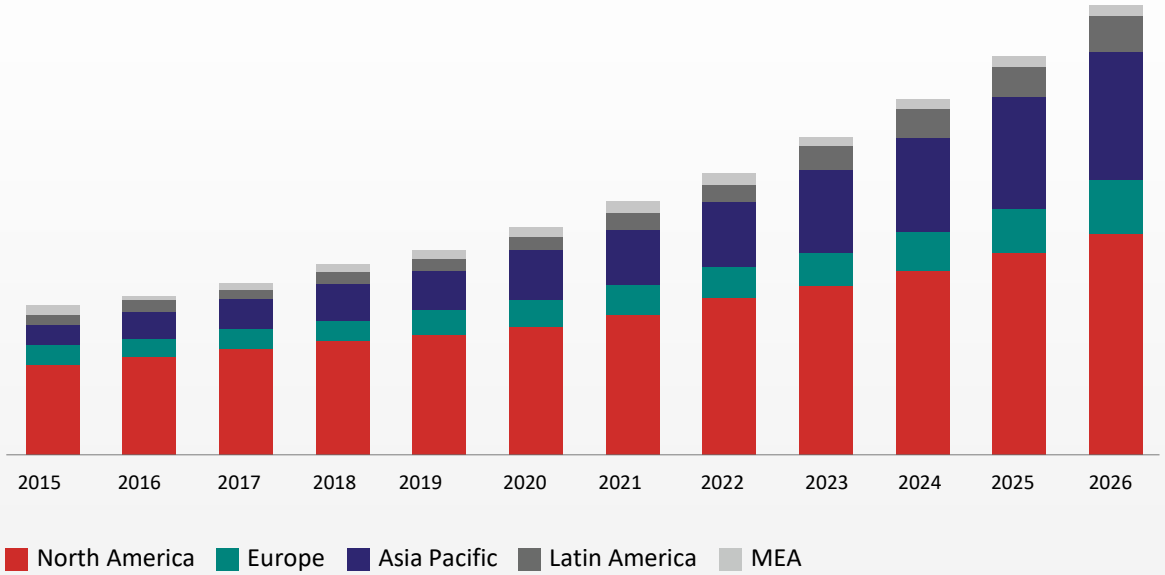
Only about 5% **of rare diseases** have an FDA-approved drug treatment[1]

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; CDER, Center for Drug Evaluation and Research; MEA, Middle East and Africa.

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

Costs Associated With Rare Diseases Have Increased Exponentially[1]

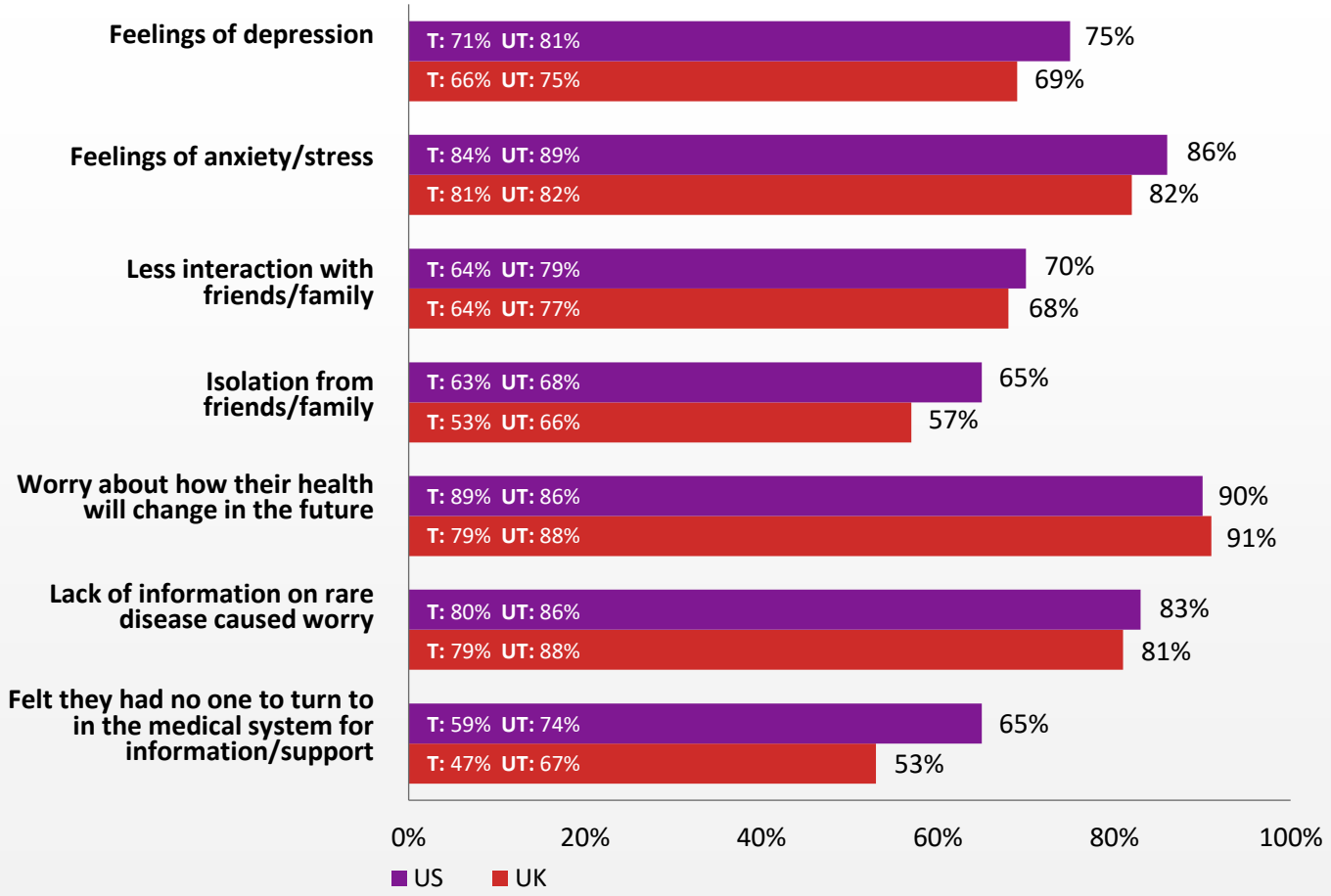
Economic impact[1]

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** range between **$600K** and **$1.5M** respectively, plus **$50k** to **150K** annually in associated costs[3]

Emotional impact[4]



*An orphan drug is a pharmaceutical agent developed to treat medical conditions, which, because they are so rare, would not be 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 **2.** Every Life Foundation for Rare Diseases. Accessed April 2022. https://everylifefoundation.org/wp-content/uploads/2021/02/The_National_Economic_Burden_of_Rare_Disease_Study_Summary_Report_February_2021.pdf **3.** Data on file. Rocket Pharmaceuticals. 2022. **4.** Global Genes. Accessed April 2022. https://globalgenes.org/wp-content/uploads/2013/04/ShireReport-1.pdf

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

Addressable Market – 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 Study: Treatment Completed

Non-randomized open label study in male DD patients

Pediatric
8 to 14 years
n=2 at CHOP

Adults (and Adolescents)
≥15 years
n=5 at UCSD

Single intravenous dose of RP-A501 (AAV9.LAMP2B) delivering full coding sequence of the *LAMP-2B* gene

****Enrollment Complete****

Cohort	Patient ID	Age at infusion	Time of follow-up (months)
Low dose ($6.7x10^{13}$ GC/kg) Pediatric	1008	12.3	12
	1009	11.7	6
Low dose ($6.7x10^{13}$ GC/kg) Adult and older adolescent	1001	17.4	36
	1002	20.3	36
	1005	18.3	30
High dose* ($1.1x10^{14}$ GC/kg) Adult and older adolescent	1006	21.1	24
	1007	20.7	N/A†

6 to 36 months

PRIMARY OUTCOMES

- **Early and long-term safety**
- Target tissue transduction and LAMP2B protein expression
- Improved myocardial histology
- Clinical improvement or stabilization

Data Reporting Details

- Pre-dose (baseline) value defined as the mean values from all visits prior to infusion
- Core lab data presented for echocardiographic parameters, cardiac serologies and cardiac histology

CHOP, Children's Hospital of Philadelphia; DD, Danon disease; *LAMP-2B*, lysosome-associated membrane protein 2B; UCSD, University of California San Diego.
*No further enrollment at this dose. †Due to advanced heart failure at the time of dosing (LVEF <40%), patient 1007 received a heart transplant 5 months following infusion of RP-A501. Patient is currently stable

RP-A501 Demonstrates Favorable Safety Profile With Enhanced Immunomodulation Protocol

ADULT COHORT

All SAEs observed within initial 2-4 months following dosing; reversible with supportive care

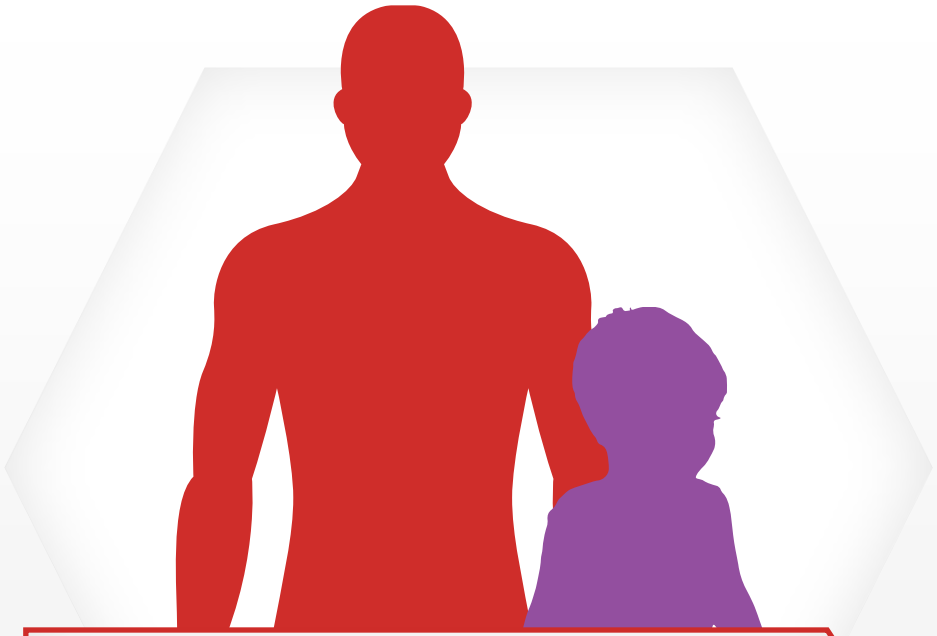
Low Dose

- **No SAEs related to drug product:** 2 steroid related SAEs (myopathy)

High Dose

- One instance of reversible TMA; led to enhanced RMP
- One instance of steroid myopathy

- **Both high and low-doses continue to be well tolerated at 2-3 years post treatment**
- **No additional SAEs observed following initial 2-4 months**



Revised Immunomodulatory Protocol:

- More rigorous daily monitoring of labs in initial days following infusion with independent clinical review team
- Reduced steroid dose with earlier taper
- Administration of sirolimus and rituximab

PEDIATRIC COHORT
(Low dose)

No RP-A501–related SAEs

All AEs were transient and reversible, with 8 and 13 months of follow up in 1008 and 1009, respectively

Platelets remained within normal range

No reported **skeletal myopathy or late transaminitis** with initial steroid dose reduction and more rapid taper, and introduction of sirolimus

- **Minimal complement activation**
- **No complement-related clinical or laboratory AEs**
- **All AEs were transient and reversible**
- **No treatment-related SAEs**

AE, adverse event; SAE, serious AE; TMA, thrombotic microangiopathy.
Data cut-off September 27, 2022, with source data verification through July 11, 2022.

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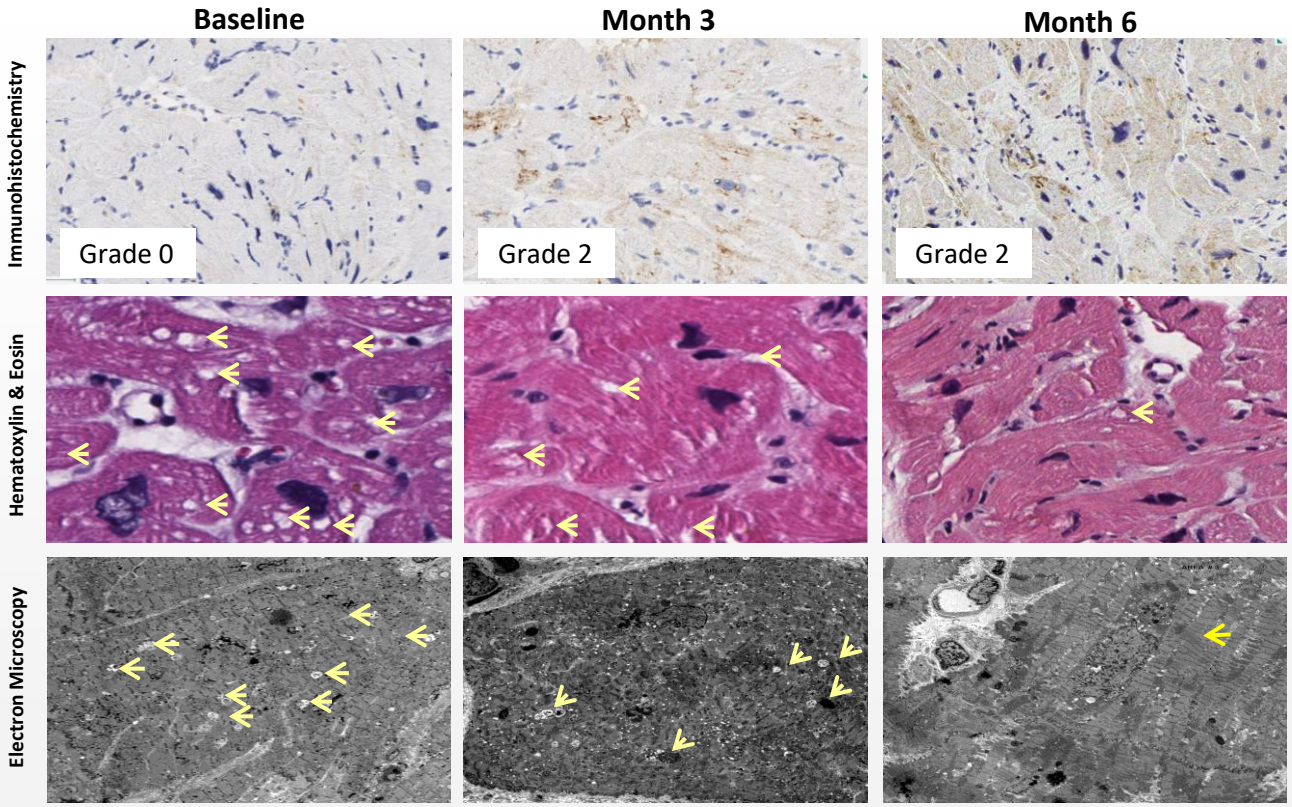
LAMP2 Myocardial Protein Expression and Histologic Improvement in the Pediatric Cohort

A501-008-1008 Endomyocardial Biopsy (EMB) Images



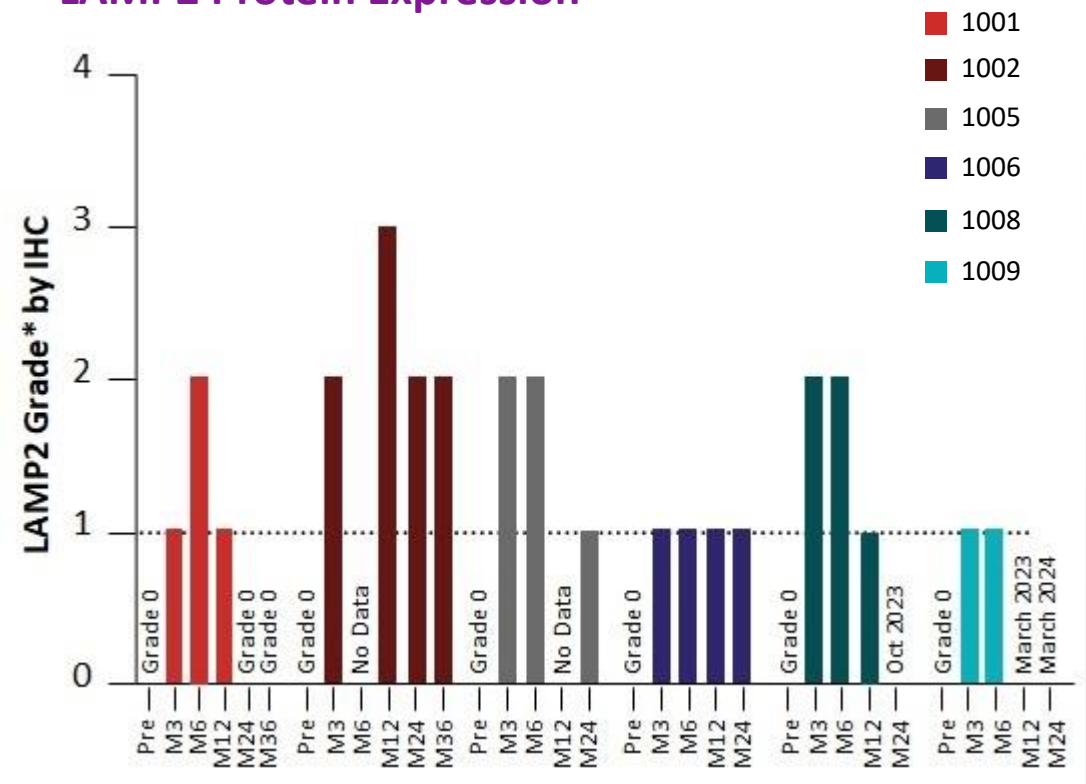
LAMP2 protein expression assessed (relative to normal human controls) by core lab in a blinded fashion of entire tissue sample

- Percentages reflect estimated extent of LAMP2 staining
  - Grade 0 negative staining
  - Grade 1 ≤25%
  - Grade 2 26%-50%
  - Grade 3 51%-75%
  - Grade 4 >75%
- H&E images captured at 20x magnification, presented digitally zoomed
- Arrows indicate autophagic vacuoles
- Similar findings on EMB from patient 1009 at Baseline and Month 3

EMB, endomyocardial biopsy; H&E, hematoxylin & eosin stain; LAMP2, lysosome associated membrane protein 2.

Pediatric LAMP2 Protein and DNA Suggests Durable Expression As Demonstrated in Adult Cohort

LAMP2 Protein Expression



*LAMP2 protein expression assessed (relative to normal human controls) by core lab in a blinded fashion of entire tissue sample; Percentages reflect estimated extent of LAMP2 staining: Grade 0=negative staining; Grade 1 ≤25%; Grade 2 =26%-50%; Grade 3 =51%-75%; Grade 4 >75%.

Cardiac LAMP2 DNA by qPCR
(vector copies per diploid nucleus)

Patient ID	Predose	Month 6	Month 12	Month 36
1001†	0	0.384	0.197	0.120
1002	0	ND	0.575	0.590§
1005	0	0.583	ND	1.228§
1006	0	2.693	1.131	-
1008	0	0.492	-	-
1009	0	Data pending	-	-

Note: Cardiomyocytes frequently multinucleated and/or have polyploid nuclei (several genome copies per cell); however, VCN is calculated assuming one diploid nucleus per cell. As a result, presented VCNs likely underestimated by factor of 2-4[1]; ND. not done, -, visit pending.
† Corticosteroid compliance uncertain. § Month 30 visit.

LAMP2, lysosome associated membrane protein 2; M, month. IHC = immunohistochemistry; qPCR = quantitative polymerase chain reaction; ND = not done.
The 2nd Cohort 2 pt (1007) underwent heart transplant due to DD progression five months post RP-A501 infusion; data post not included. Pt is currently clinically stable.
[1]Derks W, Bergmann O. Polyploidy in Cardiomyocytes: Roadblock to Heart Regeneration?. Circ Res. 2020;126(4):552-565.

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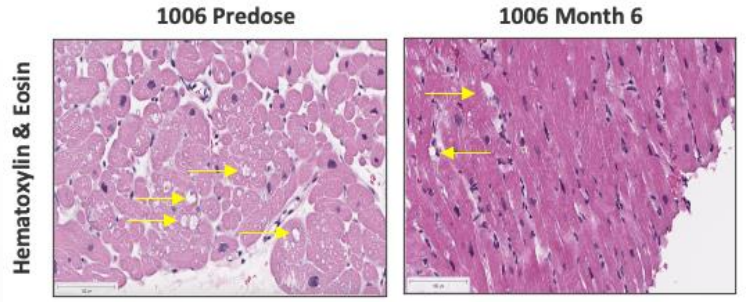
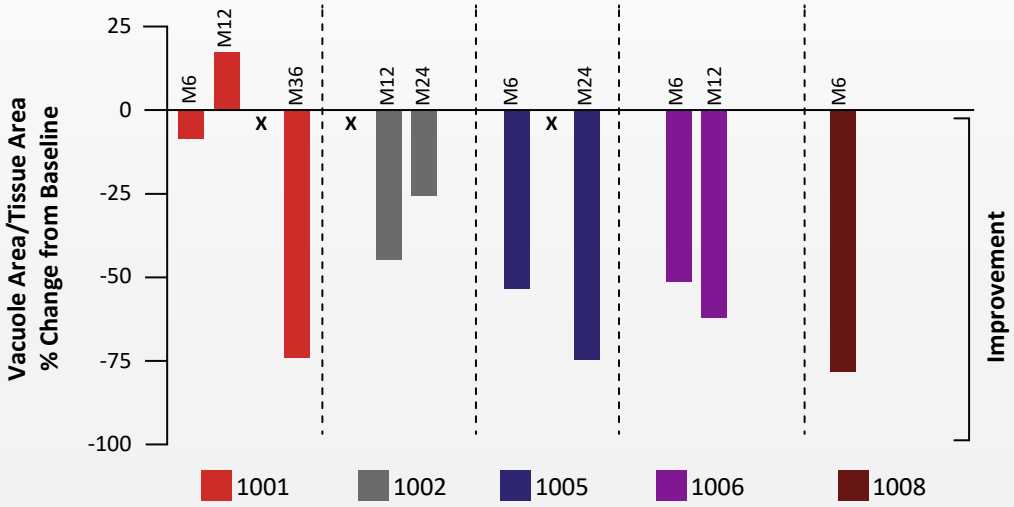
Restored Autophagy is Sustained Following RP-A501

Restored autophagy indicated by attenuation of vacuolar area

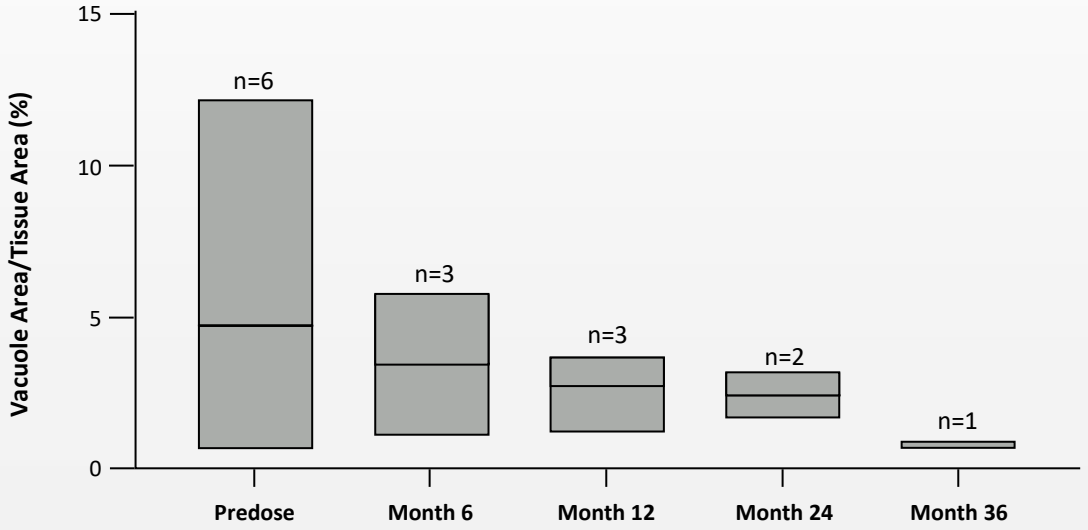


Light microscopy images at 20X;
Autophagic vacuoles are depicted by yellow arrows.

A. Vacuolar Area of Endomyocardial Tissue



B. Vacuolar Area Decreases with Treatment



M, month; X, data not assessed

Bars denote minimum and maximum range, and line within each bar represents the mean value within study population.

Improvement or Stabilization Observed Across Key Biomarker, Echo Findings and Functional Measures in Phase 1 RP-A501 Study

Cohort	Patient ID	Duration of Follow-up (months)	Myocardial LAMP2, (IHC grade*)	hsTnI (ng/mL)	BNP (pg/mL)	LV mass (g)	Max LV Wall Thickness (mm)	NYHA class	KCCQ score
Low dose adult/ adolescent	1001	36	1	0.6 ⇢ 0.01 (-98%)	55 ⇢ 59 (+8%)	311 ⇢ 212	25 ⇢ 23	II ⇢ II	44 ⇢ 49
	1002	36	3	1.46 ⇢ 0.06 (-96%)	1308 ⇢ 80 (-94%)	989 ⇢ 511	64 ⇢ 38	II ⇢ I (M30)	64 ⇢ 81
	1005	30	2 (M6)	0.28 ⇢ 0.15 (-46%)	166 ⇢ 176 (+6%)	438 ⇢ 375	33 ⇢ 24	II ⇢ I	77 ⇢ 85 (M24)
High dose adult/ adolescent	1006	24	1	0.54 ⇢ 0.20 (-63%)	135 ⇢ 42 (-69%)	410 ⇢ 300	22 ⇢ 18	II ⇢ I	79 ⇢ 82
Low dose pediatric	1008	12	1	1.89 ⇢ 0.26 (-86%)	1837 ⇢ 306 (-83%)	605 ⇢ 432 (M9)	42 ⇢ 36 (M9)	II ⇢ I	50 ⇢ 82
	1009	6	1 (M6)	0.67 ⇢ 0.07 (-90%)	297 ⇢ 113 (-62%)	234 ⇢ 185	20 ⇢ 20	II ⇢ I	52 ⇢ 78

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.
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 July 11, 2022; *at month 12 assessment, or earlier if m12 not available; Grade 0=negative staining; Grade 1 ≤25%; Grade 2 =26%-50%; Grade 3 =51%-75%; Grade 4 >75%.

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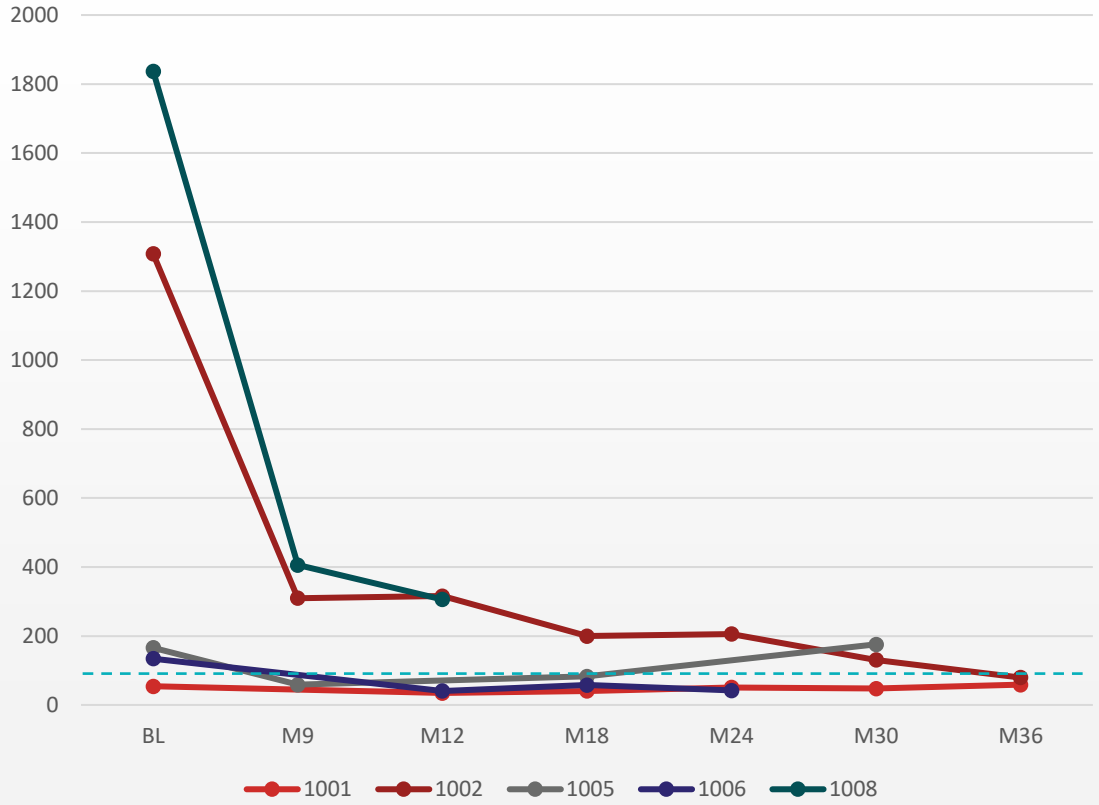
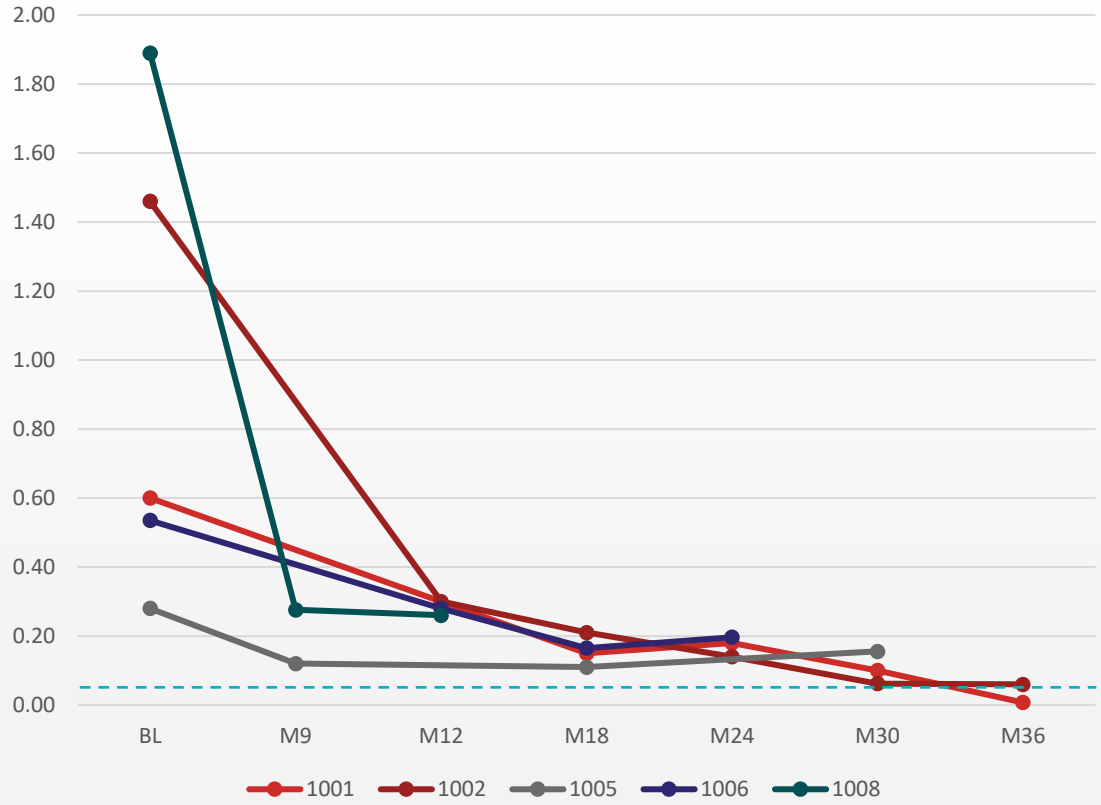
Improvement or Stabilization Observed Across Key Cardiac Biomarkers

BNP (pg/mL)



Troponin-I (ng/mL)



Indicates normal range (>100pg/mL BNP; >0.04ng/mL Troponin-I)

Note: Graphs do not depict serologic values during initial months after therapy; BL, baseline; M, month. 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.

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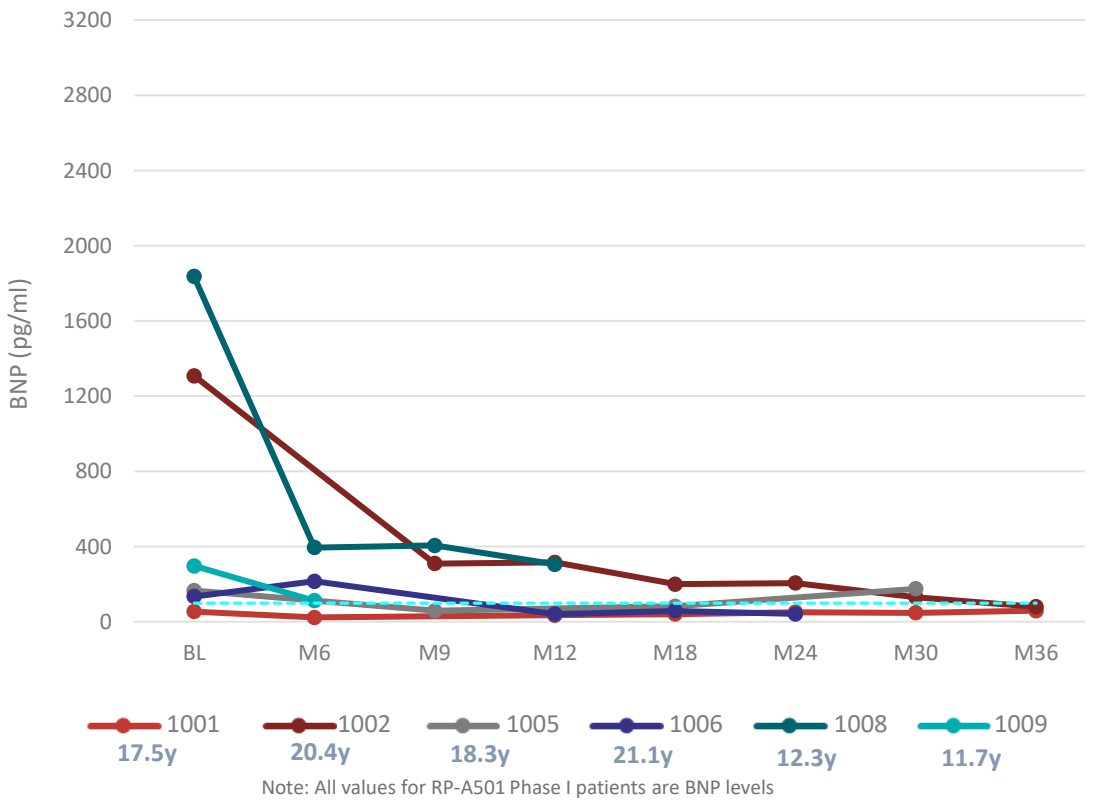
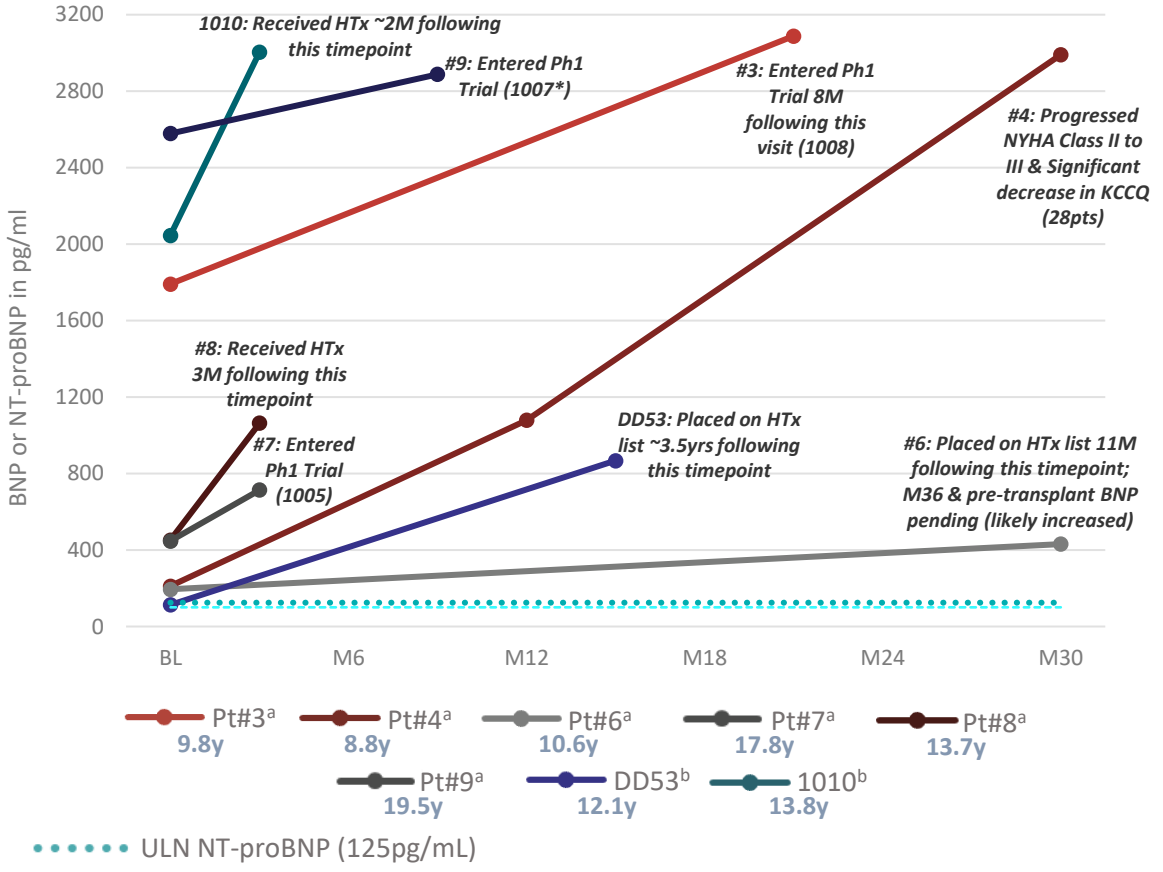
RP-A501 Phase 1 Patients: Marked Divergence from Natural History in Key Biomarkers

RP-A501 Phase 1
Baseline & Timepoints ≥6 months



Note: All values for RP-A501 Phase I patients are BNP levels

Sample Pediatric and Adolescent External Comparators



Note: [a]NT-proBNP tends to be higher than corresponding [b]BNP values given longer plasma half-life. External comparator data from prospective natural history (#s 3,4,6,7,8,9), natural history (DD53) and screen failure from Phase I study (1010). Phase I graph only reflects BNP levels and does not depict serologic values prior to initial 6 months after therapy. 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.

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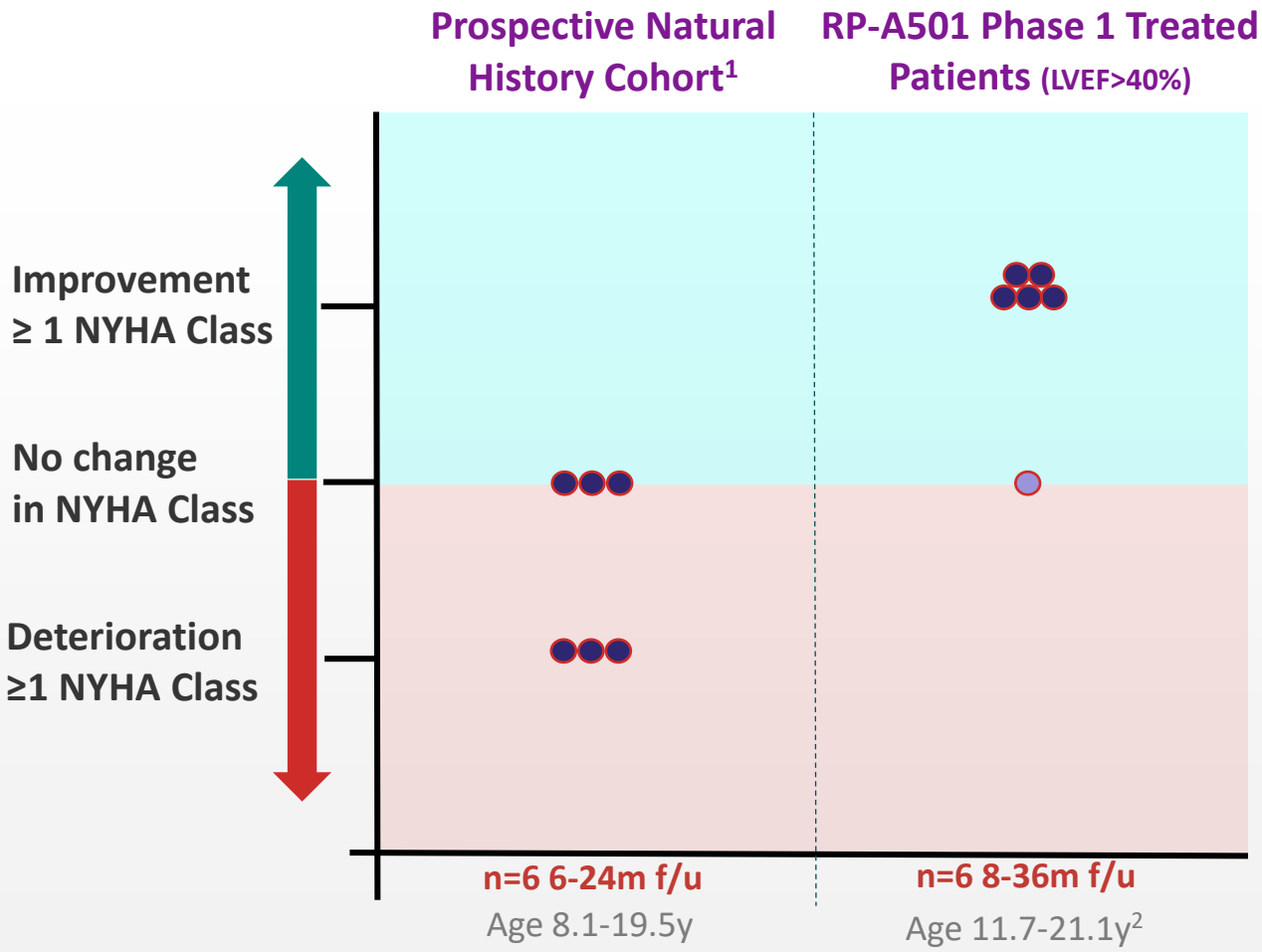
NYHA Class in Danon Disease Male Patients:
Natural History versus RP-A501 Phase 1



Prospective Natural History:
- **No patients had improved NYHA Class**

RP-A501:
- **All patients** with baseline LVEF ≥40% and monitored immunomodulation **had improved NYHA Class** (from Class II at baseline to Class I)

- Indicates pts with LVEF ≥40% at enrollment in prospective Nat Hx study or RP-A501 Phase 1
- Indicates pts with LVEF ≥40% at enrollment in RP-A501 Phase 1; unmonitored immunomodulation

Note: 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.
[1]Prospective natural history cohort: Sequential NYHA information was available for 6 of 9 patients in cohort. For remaining 3 patients, 1 was lost to follow-up, one received heart transplant (presumably deteriorated), and one was enrolled in the Phase 1 study.
[2]RP-A501: Pediatric patients age 11.7 and 12.3 years at rx; all other pts age 17.4-21.1 years at rx.

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. -**Pt 1005**

Prior to therapy, he was afraid of dying and wanted a chance at life……After gene therapy, we see him smile more now, he bought his own place and working a couple of days a week, he has started to open up for meeting more friends in real life and has gotten a whole new peace of mind now …he feels better, and he didn't think that would ever happen –**Pt 1006**

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

He walked a 10K with his father following treatment. He is exercise training twice a week for an hour. -**Pt 1009**

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Projected Pivotal Study Design

Key Agreements Reached with FDA

- ✓ $6.7x10^{13}$ GC/kg dose
- ✓ Single-arm, open-label study (randomization not appropriate)
- ✓ Support for use of natural history as external comparator information
- ✓ Potential for accelerated approval based on a composite biomarker-driven endpoint
- ✓ 6MWT, CPET are not appropriate endpoints in DD

Elements in Discussion

- Specific components of composite endpoint including LAMP2 expression
- Trial duration and time to endpoint
- 2 patient run-in for pediatric enrollment (age 8-14 years)

Confirmation pending submission of Phase II protocol and FDA review

Additional Study Elements

- Will utilize revised Phase I eligibility criteria (i.e. LVEF >50%)
- Age 8 years and older
- Optimized immunomodulatory regimen used in Phase I pediatric cohort
- All drug product will be produced in-house at Cranbury, NJ facility

Based on End of Phase I Regulatory Discussion and ongoing dialogue with FDA.

Robust Ongoing Natural History Efforts to Support External Comparator Sample

Prospective Natural History Study

9 Males + 1 Female

Initiated in 2019, expansion planned

- 2 underwent HTx
- 1 with low LVEF at enrollment progressed rapidly to end stage
- 4 referred to RP-A501-0219
  - 3 enrolled
  - 1 not enrolled (Phase 1 enrollment closed)
- 5 in follow-up

+

US Retrospective Natural History

51 Males + 67 Females

Initiated in 2006

- 13 HTx
- 7 died
- 9 HTx
- 1 died

+

Literature review
(Brambatti 2019 publication*)

+

EU Retrospective Natural History Study

Males **(goal 60)** + Females **(goal 60)**

Initiated in 2022

HTx, heart transplant; LVEF, LVEF, left ventricle ejection fraction.
*Brambatti M, Caspi O, Maolo A, et al. Danon disease: Gender differences in presentation and outcomes. Int J Cardiol. 2019;286:92-98.

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 likely enhances safety profile

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 in 2023

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 drug.

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Development Plan

Moving toward pivotal global Phase 2 study

Study Milestones

- ✓ Phase 1 treatment completed in males
- ✓ RMAT, Orphan Drug, Rare Pediatric and Fast Track designations in the US (PRV eligible)
- ✓ Completed 2 in-house cGMP batches
- ✓ End of Phase 1 Regulatory Meeting held with FDA

Ongoing Activities

- Final Phase 2 Study Design and Endpoints
- Initiate Phase 2 Global Pivotal Study Activities
- Expanded natural history study

GLOBAL REGISTRATIONAL PHASE 2 STUDY

FDA, Food and Drug Administration; RMAT, Regenerative Medicine Advanced Therapy, PRV, priority review voucher.
Data on file. Rocket Pharmaceuticals. 2023.

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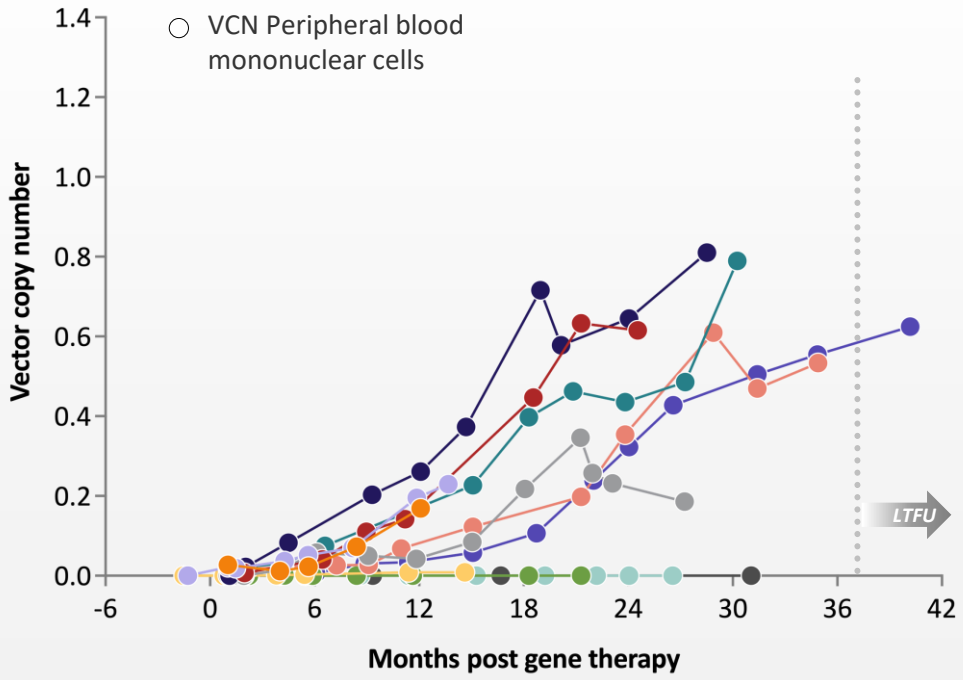
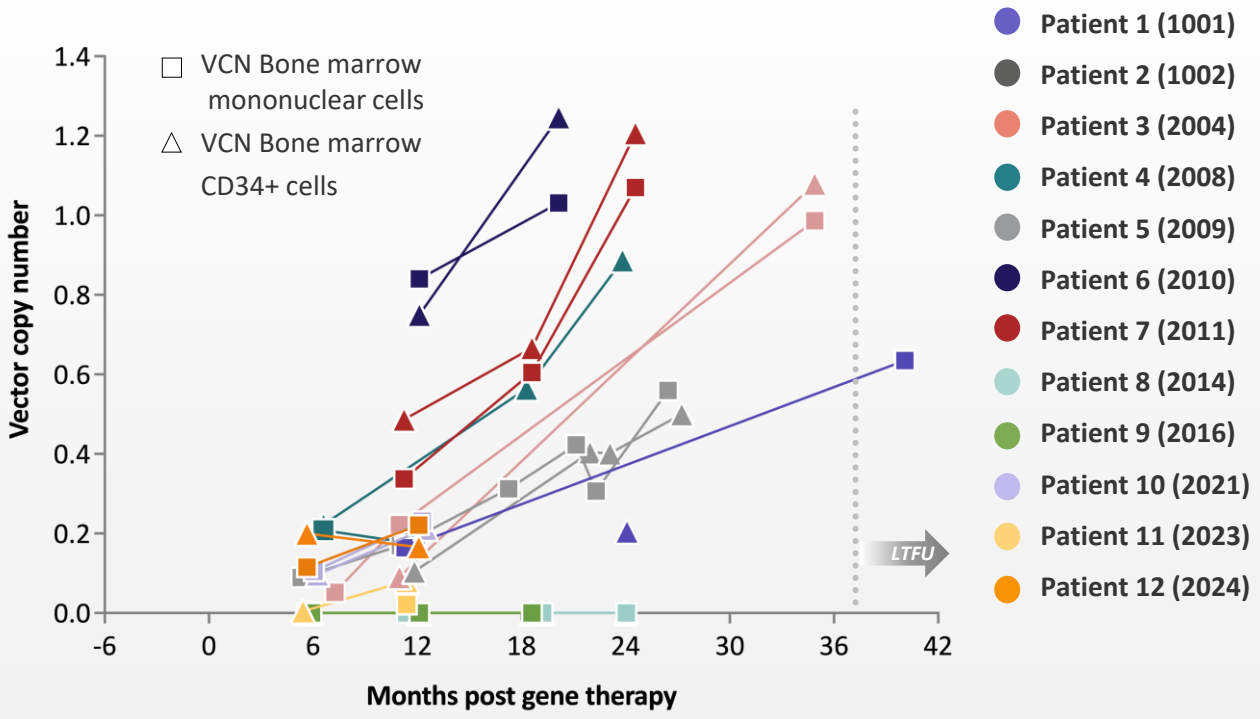
Progressively Increasing and Sustained Genetic Correction in 8 of 12 Patients ≥1 Year Post–RP-L102

Progressive increases in gene markings in PB and BM in 8 patients

PBMC VCN



BM VCN



BM, bone marrow; CD34, cluster of differentiation 34; 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: April 17, 2023; Preliminary interim results are presented from the ongoing clinical studies.

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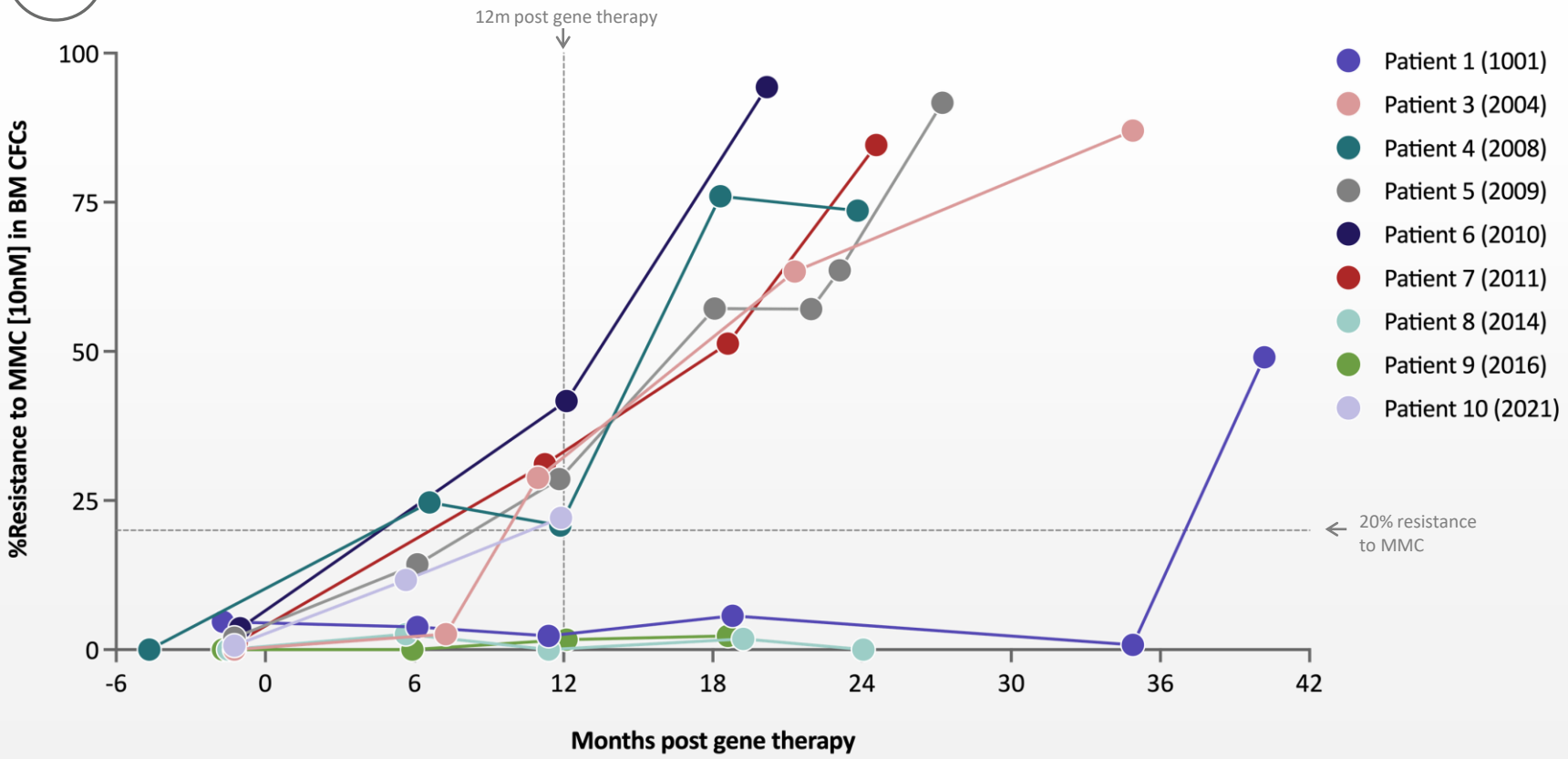
Increasing Phenotypic Correction (MMC-resistance) over 1 to 3 Years Post–RP-L102

Sustained BM CFC MMC resistance observed in at least 6 patients*



For 5 patients, increased BM CFC MMC resistance ranging from 51% to 94% was observed at 18 to 24 months post–RP-L102 administration

MMC resistance of >20% achieved at 2 consecutive timepoints ≥12 months for n=5

BM, bone marrow; CFC, colony-forming cells; MMC, mitomycin-C.
Not shown: MMC resistance in Patient 2 (1002), who was withdrawn from the study at 18 months post–RP-L102 infusion.
*One additional patient (Patient 1: 1001) was noted to have BM MMC resistance of 49% at ~40 months post–RP-L102 infusion.

Development Plan

Moving toward BLA/MAA filing

INITIAL EFFICACY AND HIGHLY FAVORABLE SAFETY PROFILE

- Initial comprehensive efficacy in 6/10 evaluable patients (≥12-month follow-up)
- No cytotoxic conditioning, only 1 transient RP-L102 related SAE (Grade 2)

TOP-LINE DATA READOUT ACHIEVED

- Rejection of null hypothesis with minimum of 5 patients with increased MMC resistance >10% at 2 timepoints between 12 and 36 months

NEXT STEPS

- **Update: CMC and clinical FDA discussions support BLA activities**
- 2 patients to be treated with product from commercial cell processing site in preparation for US launch

Anticipated simultaneous BLA/MAA filings

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.
Data on file. Rocket Pharmaceuticals. 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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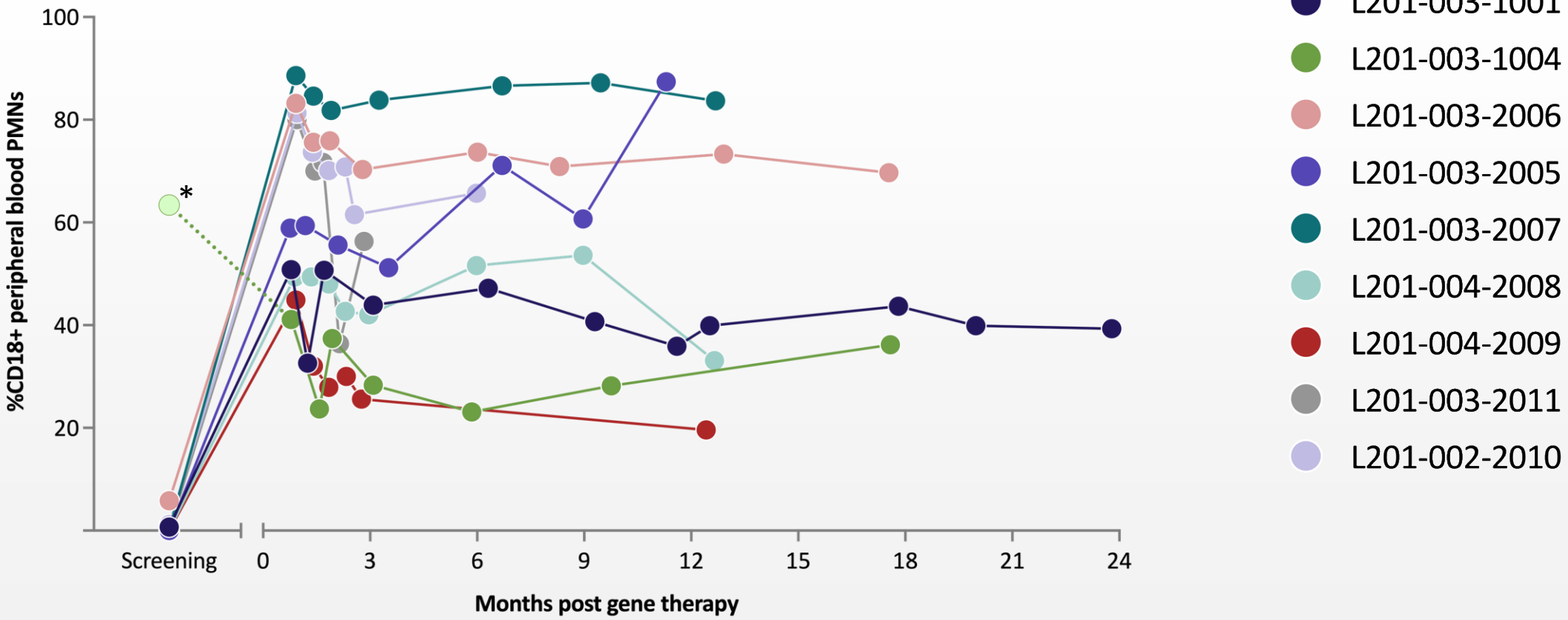
CD18 Expression in PB Polymorphonuclear Cells (PMNs)

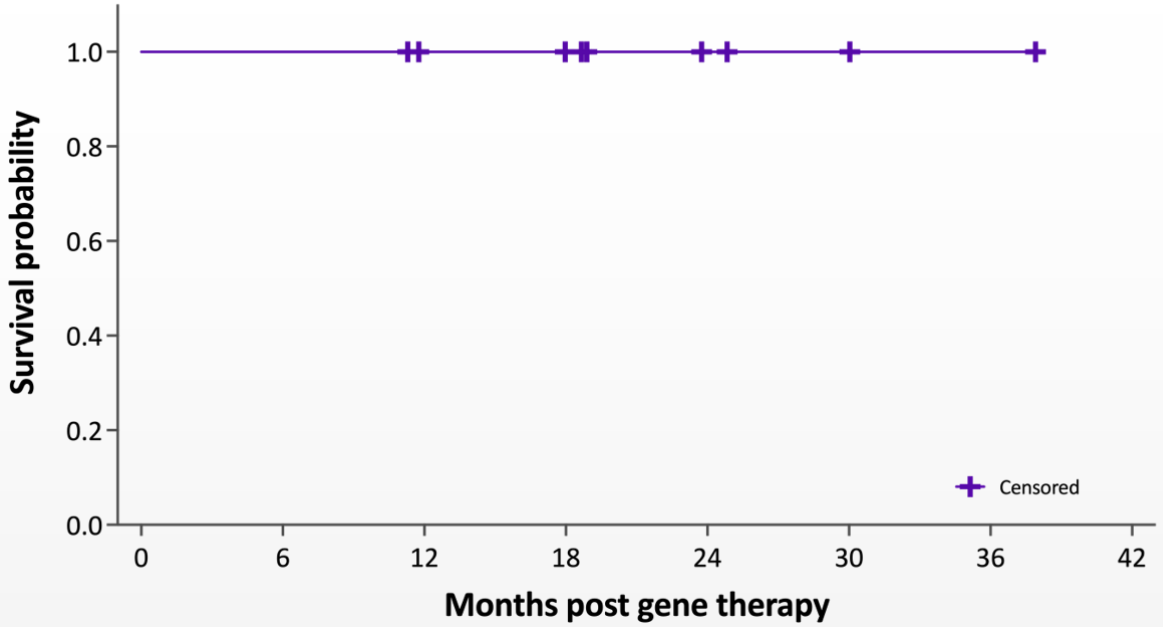
At 3 to 24 months after infusion, 9/9 patients sustained stable CD18 expression (median: 56%) with no therapy-related serious adverse events



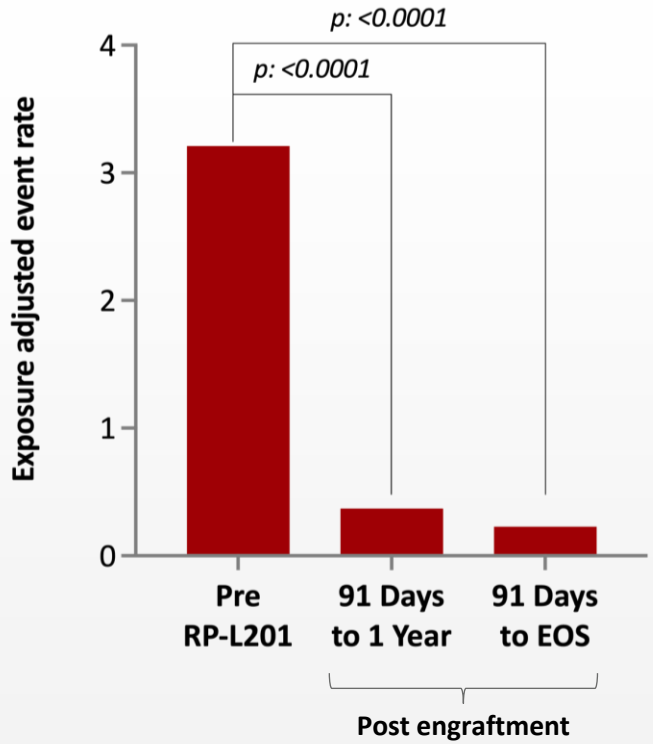
*Dim/weak CD18 expression reported at baseline for Subject L201-003-1004 in ~63% of cells in conjunction with <2% CD11a/CD11b expression, likely indicating abnormal/unstable protein.
LAD-I, Leukocyte Adhesion Deficiency-I; PB, peripheral blood; PMN, polymorphonuclear neutrophil.
Data on file. Rocket Pharmaceuticals. 2023. Data Cut-Off: April 6, 2022; Preliminary interim results are presented from the ongoing clinical study.

Significant Reduction in Hospitalizations and 100% HSCT-free Survival

100% HSCT-free survival Kaplan–Meier estimate



Significant reduction in incidence of hospitalizations^



Survival without allogeneic HSCT*

Primary outcomes

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

All patients have been able to stop prophylactic antibiotics

EOS, end of study; HSCT, hematopoietic stem cell transplantation; LAD-I, leukocyte adhesion deficiency-I.
Data cut-off: November 2, 2022,*, March 9, 2022^
Data on file. Rocket Pharmaceuticals. 2023.

Development Plan

Moving toward product filing

ENROLLMENT AND INITIAL EFFICACY

- Enrollment completed; 9/9 patients treated
- Efficacy observed in 9/9 patients with 3 to 42 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

- Progression to regulatory filing activities

Guiding Q2 2023 regulatory filing

Life-cycle management

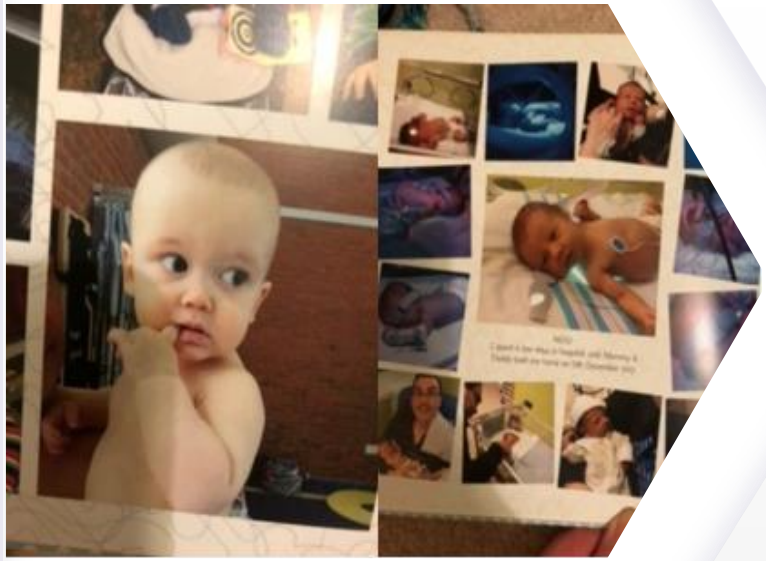
- Potential label expansion to include moderate LAD-I population
- Potential study initiation in 2023

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; GvHD, graft-versus-host disease; LAD-I, leukocyte adhesion deficiency-I; MAA, Market Authorization Application; PRIME, Priority Medicines; PRV, priority review voucher; RMAT, Regenerative Medicine Advanced Therapy; SAE, severe adverse event.
Data cut-off: November 22, 2022.
Data on file. Rocket Pharmaceuticals. 2023.

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

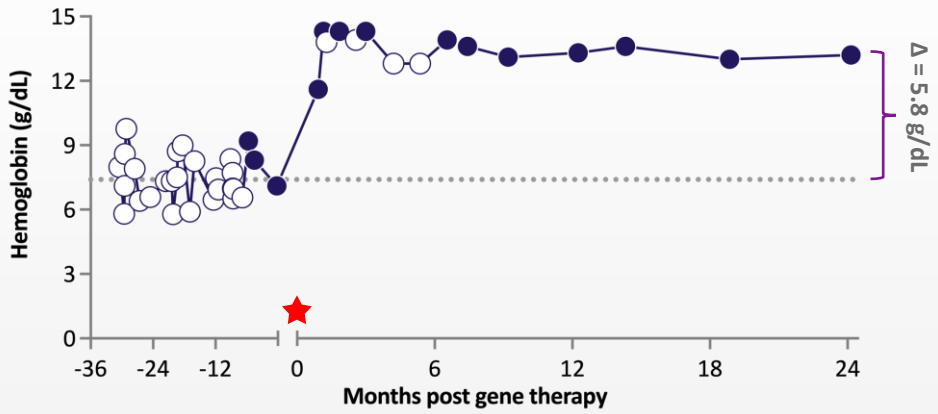
ATP, adenosine triphosphate; PKD, pyruvate kinase deficiency; PKLR, pyruvate kinase L/R; RBC, red blood cell.

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 Efficacy Results for Patients L301-006-1001 and L301-001-1002

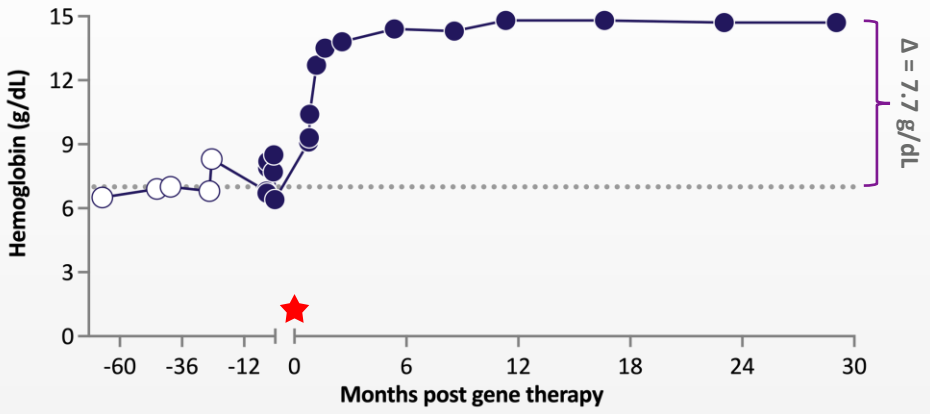
Hemoglobin improvement to normal range (from baselines in severe (<8 g/dL range)
Transfusion independence (extensive transfusion requirements prior to RP-L301)
Sustained improvement of hemolysis markers (LDH, bilirubin) and PB VCNs in 1.0 to 3.0 range

PATIENT 1001



- Sustained **hemoglobin normalization from ~7.4 g/dL to 13.2 g/dL** 24 months post–RP-L301 infusion
- No red blood cell transfusions required following engraftment

PATIENT 1002



- Sustained **hemoglobin normalization from ~7.0 to 14.7 g/dL** 30 months post–RP-L301 infusion
- No red blood cell transfusions required following engraftment

RP-L102 Infusion

..... Dotted lines indicate average hemoglobin for each patient prior to gene therapy

Assessment Performed at Clinical Site

Assessment Performed at Local Laboratory

LDH, lactate dehydrogenase; PB, peripheral blood; VCN, vector copy number.
Data cut-off: May 3, 2023; preliminary interim results are presented from the ongoing clinical study.
Data on file. Rocket Pharmaceuticals. 2023.

Development Plan

Moving toward pivotal Phase 2 study

PLAN FOR PHASE 2 AND LAUNCH

Key endpoints selected

- Hemoglobin increase
- ↓ 50% in transfusions or transfusion independence

Well-delineated natural history in recent PKD NHS publications

- Complete Phase 1 pediatric cohort dosing (N=2 to 3)
- End of Phase 1 regulatory meeting with FDA in 2023
- Approve and launch RP-L301; seek regulatory approval in the US and EU

REGULATORY DESIGNATIONS

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

LIFE-CYCLE MANAGEMENT

- Anticipated expansion study to pre-splenectomy patients
- Exploration of non-genotoxic conditioning

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. 2023.

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.
Data on file. Rocket Pharmaceuticals. 2023.

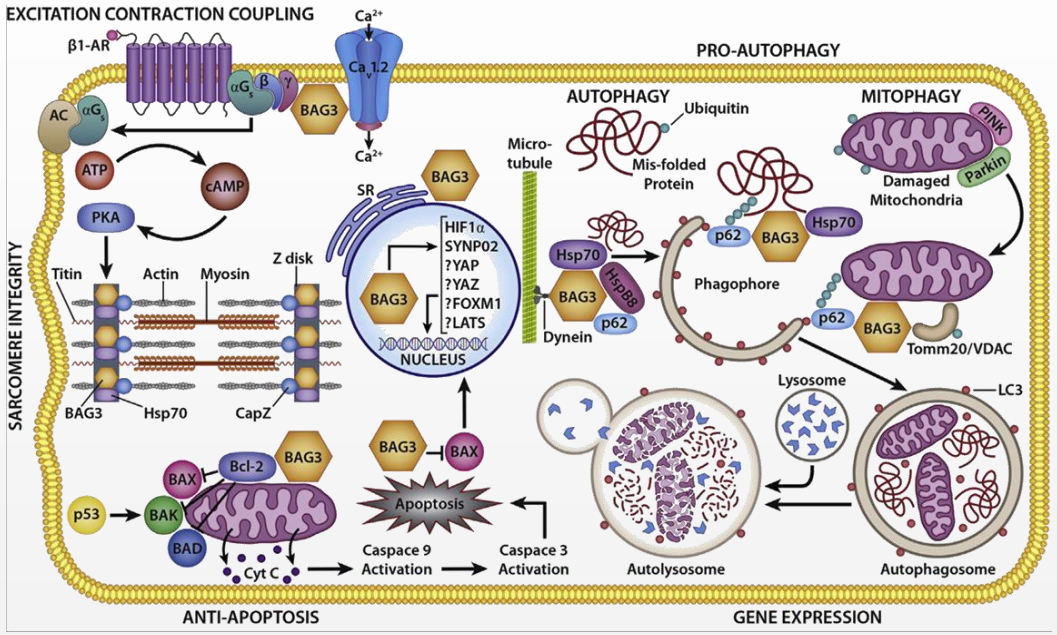
BAG3 Regulates Critical Functions in Cardiomyocytes

1 Cardiac contractility

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

2 Structural support

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

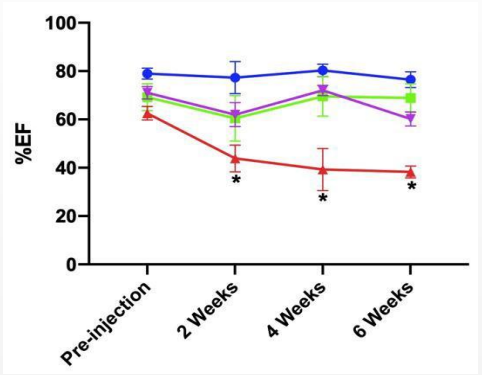
BAG3-DCM Represents a Significant Market with Unmet Need

- 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: AAV9 BAG3 treatment group

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

- Evaluating optimal development pathway; IND planned 2024

AAV, adeno-associated virus; BAG3, BCL2-associated athanogene 3; DCM, dilated cardiomyopathy; EF, ejection fraction; GFP, green fluorescent protein; H1, first half of the year; 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.

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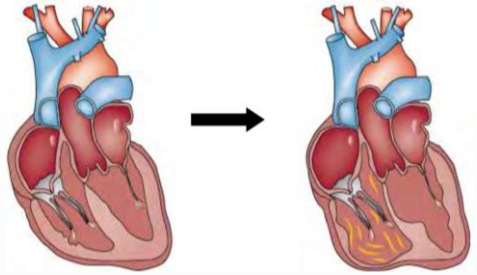
Project Pegasus (PKP2-ACM)

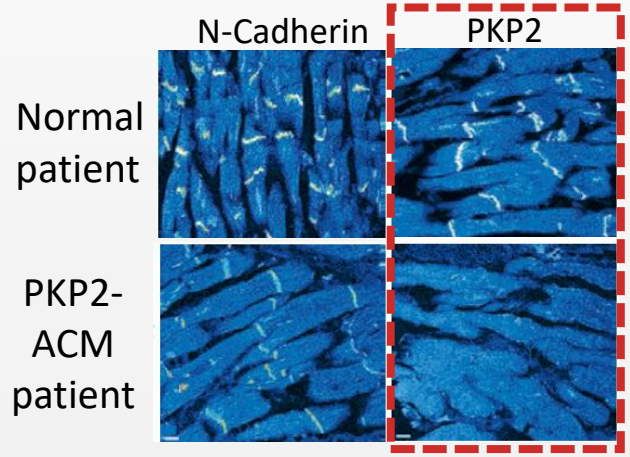
PKP2-Arrhythmogenic Cardiomyopathy (ACM)*:
A high-risk disease with no curative options

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

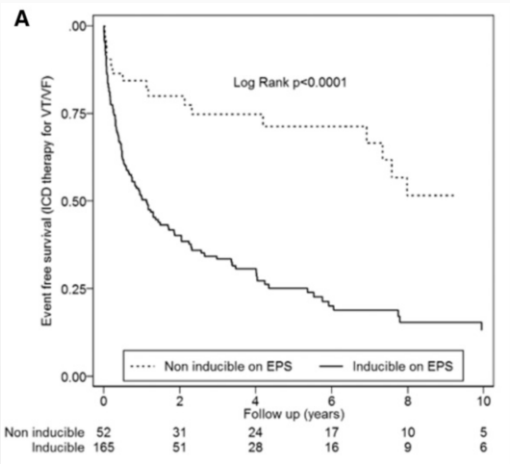
Therapeutic Challenges

- 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 pts [5]
- For pts 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]

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*

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

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.

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

Academic Partner:

NYU Grossman School of Medicine

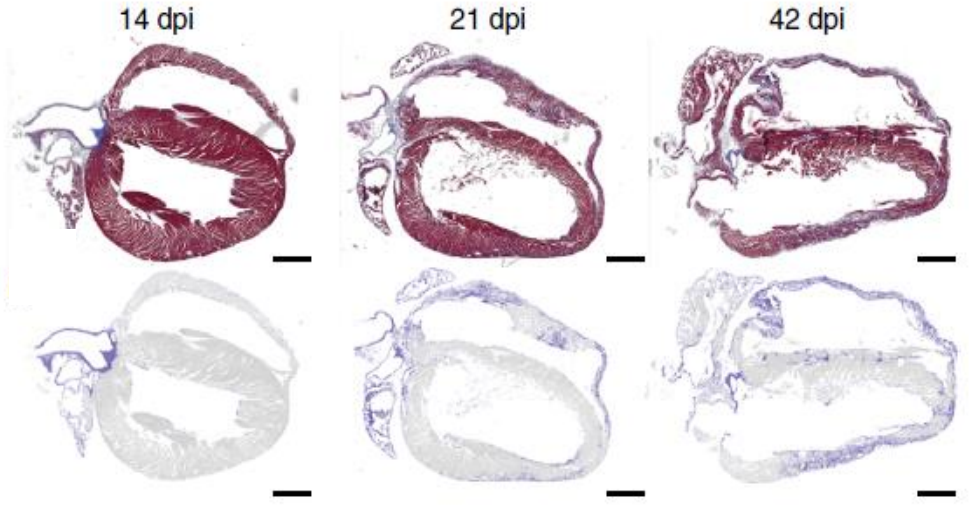
Ongoing sponsored research. No future royalty obligations

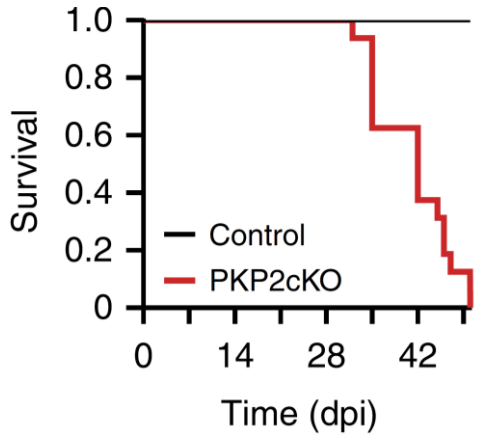
Mario Delmar, MD, PhD
Patricia and Robert Martinsen Professor of Cardiology,
Department of Medicine; Division of Cardiology,
NYU Grossman School of Medicine

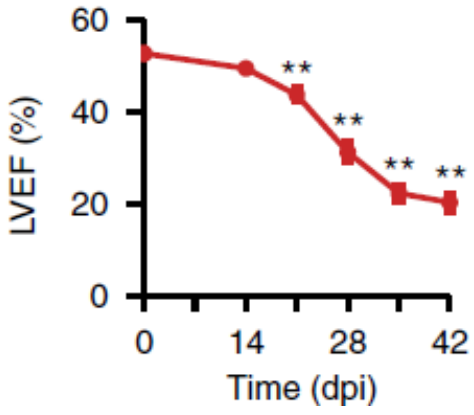
Marina Cerrone, MD
Research Associate Professor,
Co-Director, Inherited Arrhythmia Clinic,
Department of Medicine; Division of Cardiology,
NYU Grossman School of Medicine

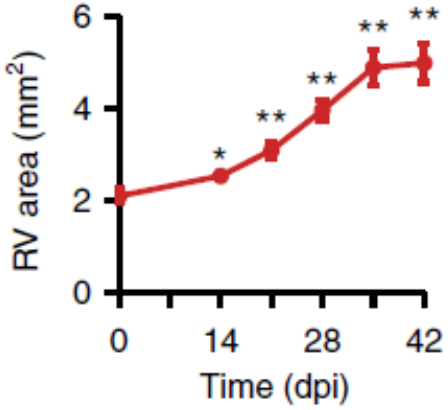
Tamoxifen-induced ACM in the PKP2-cKO Mouse Model

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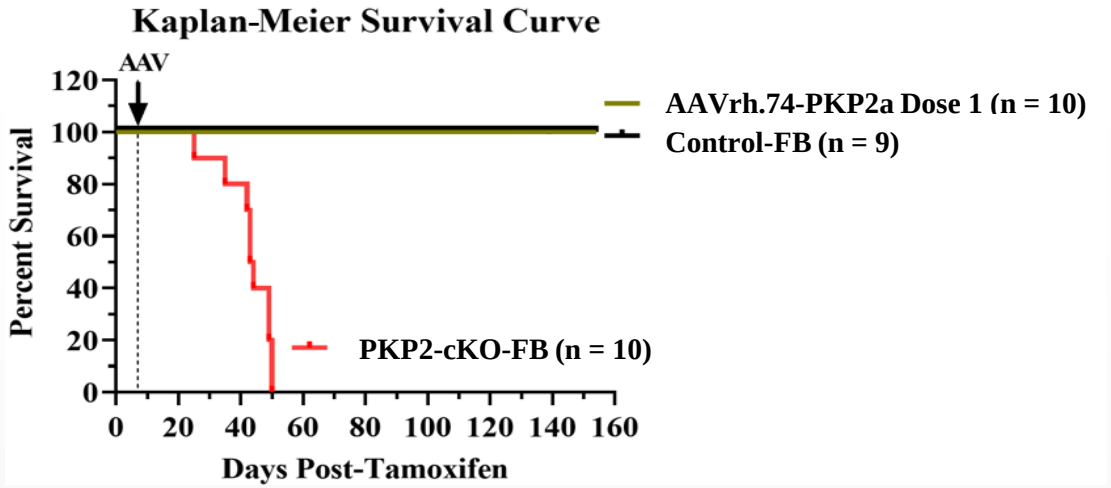
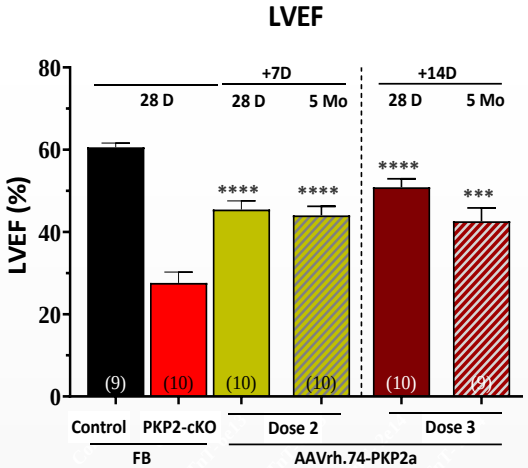
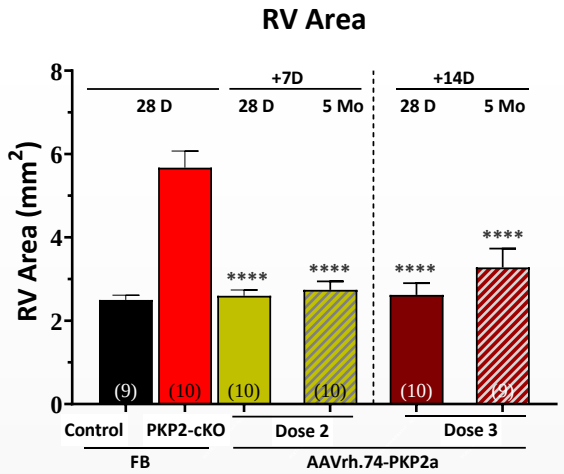
Increased Survival & Preserved Cardiac Function in the PKP2-cKO Model

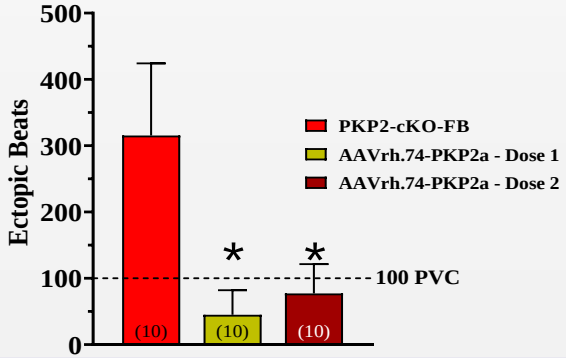


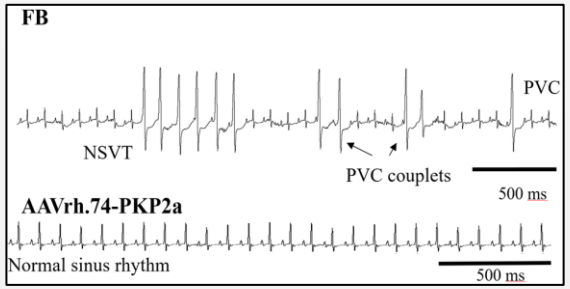




- AAVrh.74-PKP2 delivered ***7 days post-TAM***:
  - **100% survival to 5 months,** compared to 100% mortality by day ~50 in PKP2-cKO control animals
  - **Preserved Ejection Fraction and Right Ventricular Area** at 28 Days, sustained to 5 months
- AAVrh.74-PKP2 delivered ***14 days post-TAM:***
  - 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[1-2]; 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.

Clinical Development Plan

Phase 1 Dose Escalation Study

Completed or Ongoing Activities

- ✓ **IND cleared by FDA**
- ✓ 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
- Orphan Disease Designation

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; 1H, first half of the year. FIH, first in human; ICD, implantable cardioverter defibrillators

Rocket – The Leader in Rare Disease Gene Therapy

- ✓ Pre-eminent maturing gene therapy pipeline in which each program is First- and Best-in-Class
- ✓ Experienced management team with a history of delivering transformative and curative therapies to patients with devastating diseases
- ✓ Well-capitalized and poised to elevate from a clinical-stage to a commercial-stage company

Expert Leadership With Proven Track Record

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

NOVARTIS · Memorial Sloan Kettering Cancer Center · Lilly · Brigham and Women's Hospital, Founding Member, Mass General Brigham

Mark White, MB.ChB.
Chief Medical Officer, SVP
Mark is a passionate and seasoned drug developer with more than 25 years of industry experience

AstraZeneca · MedImmune

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

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

caladrius BIOSCIENCES · PCT · Celsion Corporation · Osiris THERAPEUTICS, INC

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

Pfizer · AstraZeneca · Roche · Bristol Myers Squibb · NOVARTIS

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

NewYork-Presbyterian Weill Cornell Medical Center · Mount Sinai · ImClone Systems · Lilly

Gayatri R. Rao, M.D., J.D.
Chief Development Officer of LV, SVP
7-Year former Director of FDA's Office of Orphan Products Development

FDA U.S. FOOD & DRUG ADMINISTRATION · SIDLEY

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

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Expert Leadership With Proven Track Record

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

Celgene · Andelyn Biosciences · Novartis · Merck

Monica Shah, M.D.
AAV Global Program Head, SVP
Former Director at the NIH with 20+ years experience in leading complex multi-disciplinary research programs

IQVIA · University of Maryland School of Medicine · NIH National Institutes of Health

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

Novartis · Advanced Accelerator Applications · Merck · Lilly

Peggy Speight
Head of Quality Assurance, VP
20+ years quality assurance and regulatory compliance expertise gained in pharma and at FDA

endo · Bristol Myers Squibb · J&J · FDA U.S. Food & Drug Administration

John Militello, CPA
Controller & Principal Accounting Officer, VP
20+ years of experience as an accomplished finance and accounting professional in the biotech sector

Immune Pharmaceuticals · VOLT · Retrophin · BDO

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THANK YOU!

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