



SEEKING GENE THERAPY CURES

February 2026



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Vision: Seeking Gene Therapy Cures

Values



Curiosity



Trust



Elevate

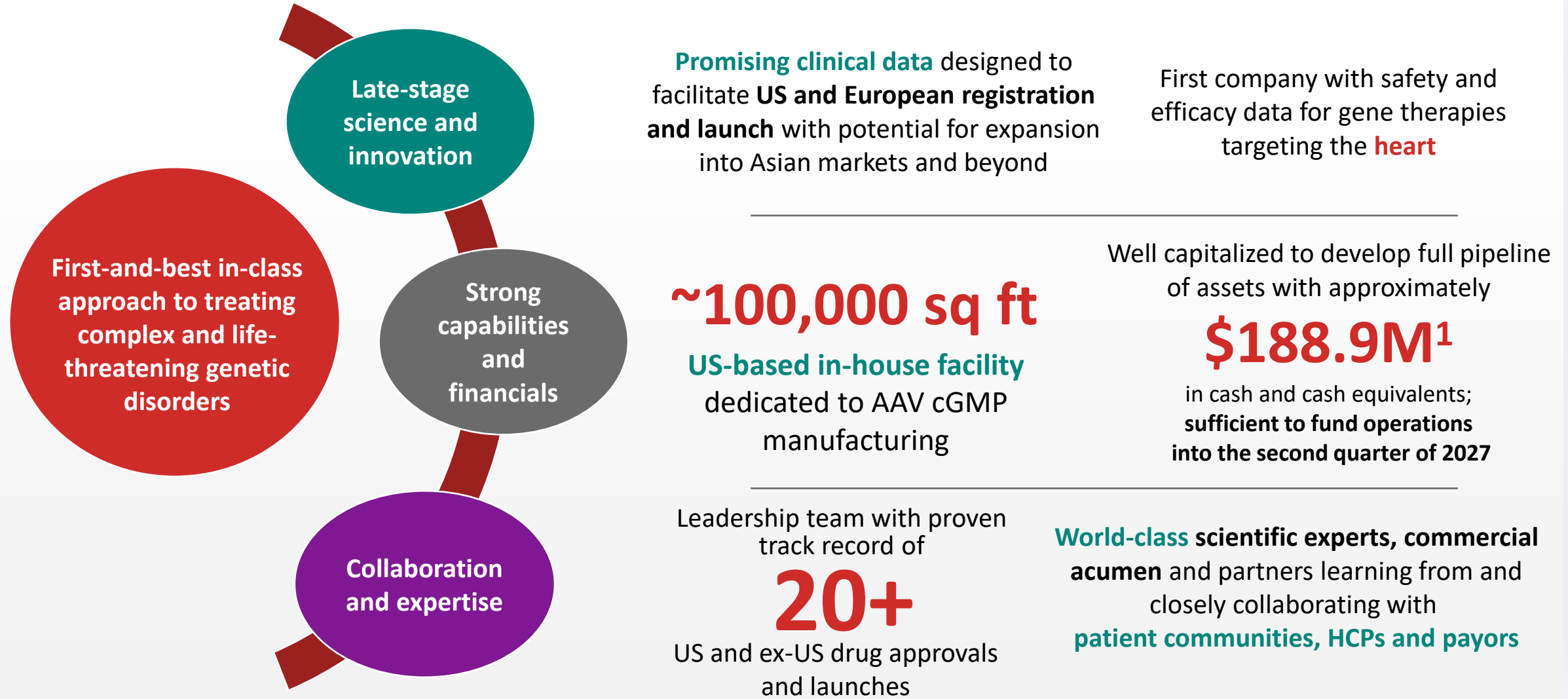


Generosity

Mission

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

A Fully Integrated Cardiovascular Rare Disease Company



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



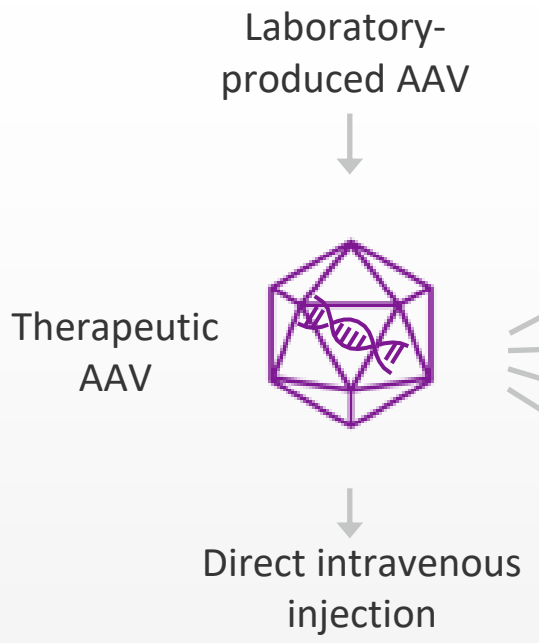
Sizeable market to maximize patient impact

THERAPEUTIC AREA	DISCOVERY	PRECLINICAL	PHASE 1	PHASE 2 (Pivotal)	Submission and Approval	DESIGNATIONS
 CARDIOVASCULAR	AAV RP-A501 Danon disease					RMAT, ATMP, Fast Track, Orphan Drug (US), Rare Pediatric, PRIME
	AAV RP-A601 PKP2-ACM					RMAT, Fast Track, Orphan Drug (US/EU)
	AAV RP-A701 BAG3-DCM					Fast Track
 HEMATOLOGY*	KRESLADI™ (LV RP-L201; marnetegrane autotemcel; marne cel)				PDUFA Date: March 28, 2026	RMAT, ATMP, Fast Track, Rare Pediatric, Orphan Drug (US/EU), PRIME
	LV RP-L102 Fanconi Anemia					RMAT, ATMP, Fast Track, Rare Pediatric, Orphan Drug (US/EU), PRIME
	LV RP-L301 Pyruvate Kinase Deficiency					RMAT, Fast Track, Orphan Drug (US/EU), PRIME
Wave 2 programs	Undisclosed Candidates					

*In its LV portfolio, Rocket is prioritizing KRESLADI™ and seeking external partnership opportunities for the RP-L102 and RP-L301 programs

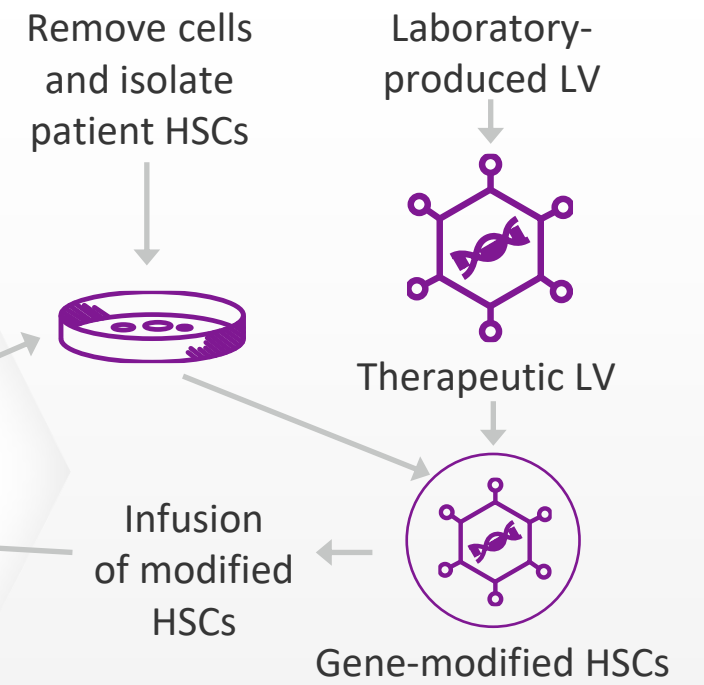
Rocket Offers Multi-platform Gene Therapy Expertise

IN VIVO platform



- RP-A501:** Danon disease
- RP-A601:** PKP2-ACM
- RP-A701:** BAG3-DCM

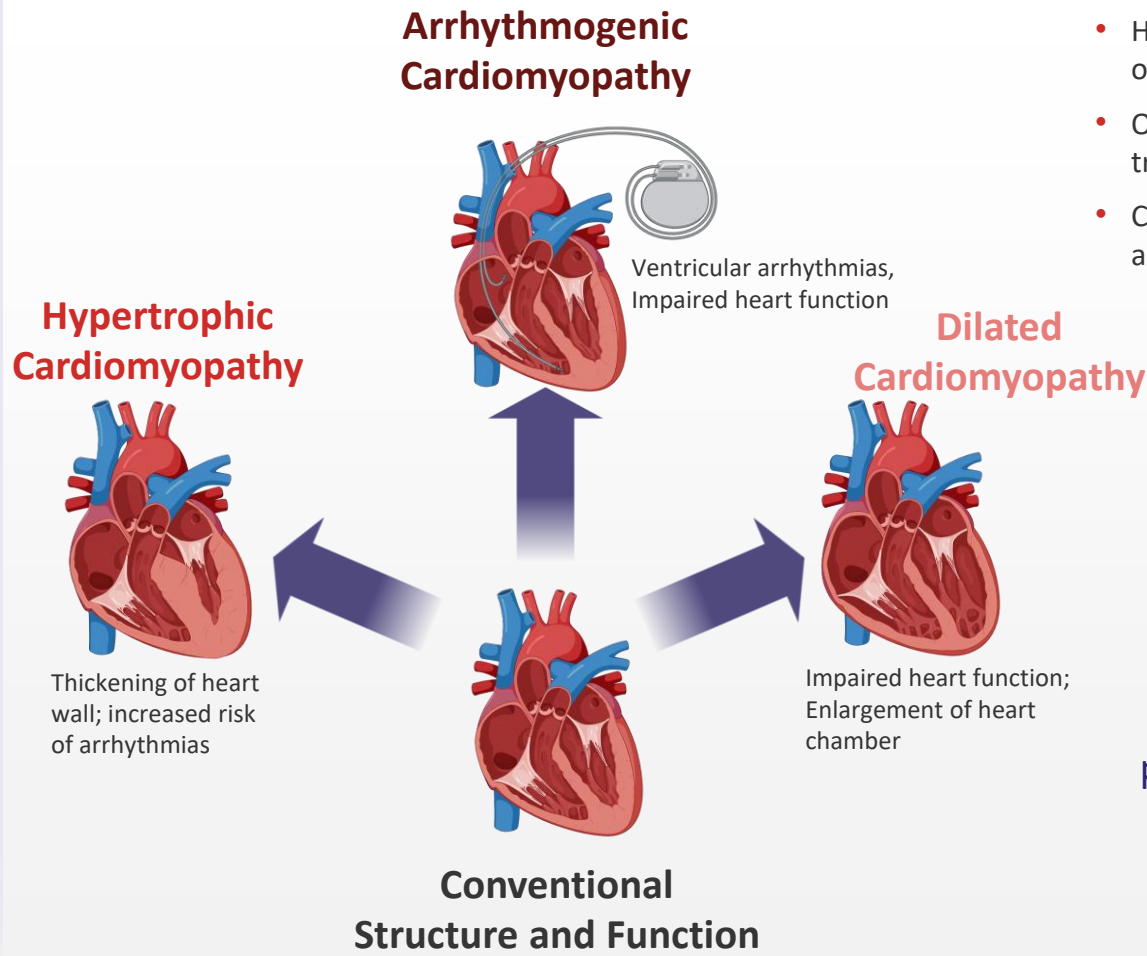
EX VIVO platform



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


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

Well-Positioned as the Leader in Cardiovascular Gene Therapies



Why Cardiomyopathy?

- High unmet medical need, current standard of care focuses on symptom palliation and does not address root cause
- One-time IV therapy with the potential to modify disease trajectory and eliminate the need for transplant
- Clear, well-established biomarkers with opportunities for accelerated approval


The average heart transplant cost in the US is ~\$1.7 million¹

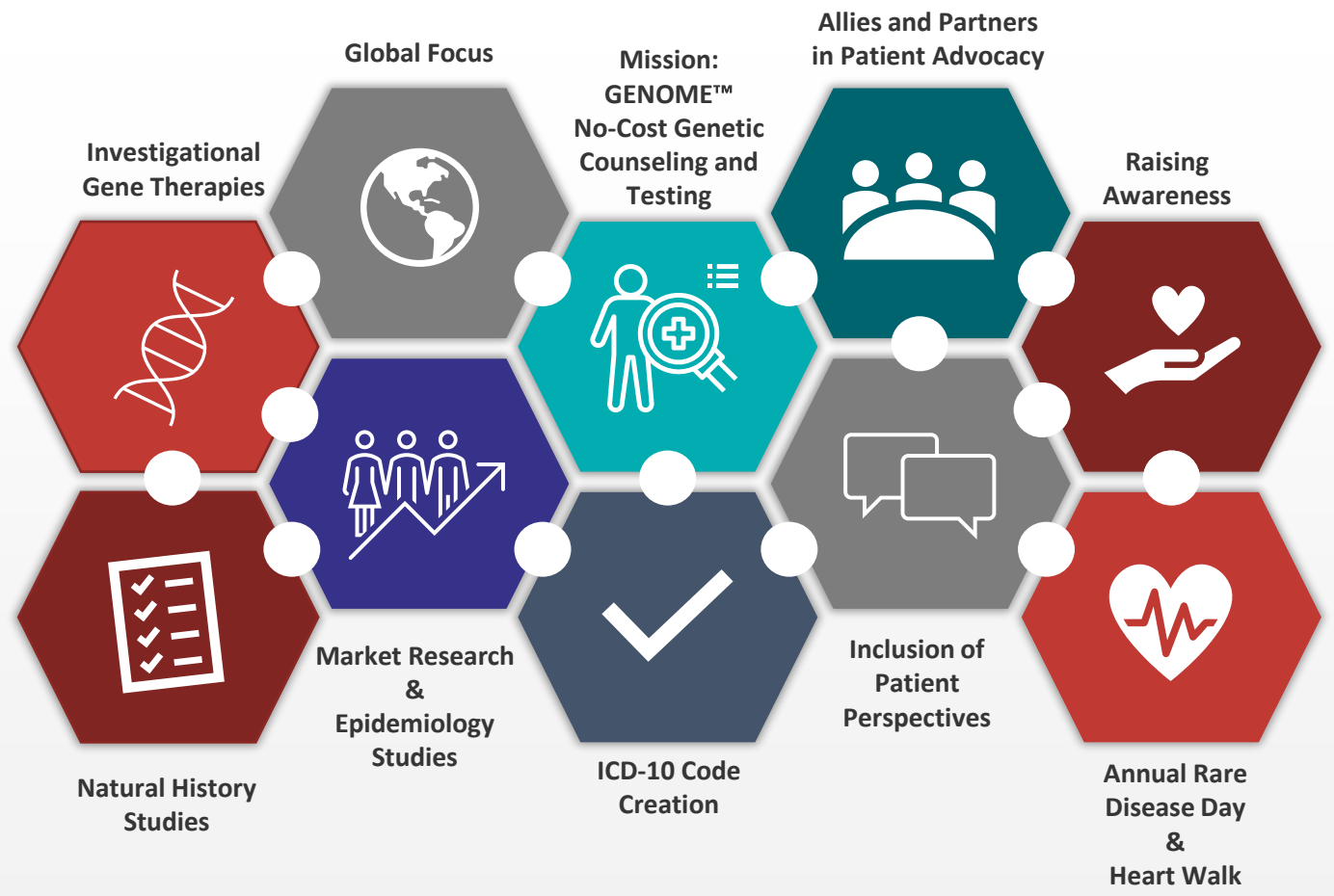
Rocket's Edge:

- First company with multi-year efficacy & safety data in cardiac gene therapy
- Proprietary AAV manufacturing
- Experience from treatment of >15 cardiac patients informs late-stage and future AAV programs
- Clinical programs focus on the major phenotypes in inherited cardiomyopathies (**HCM**, **ACM**, **DCM**), representing a market opportunity with a large unmet need with a multi-billion-dollar commercial potential



Market Potential:
Our three clinical programs account for **>100,000 patients** in the US and EU

Our Commitment to the Rare Disease Community



Our R&D and Manufacturing Capabilities

- **Total Lab Space:** ~30,000 sq. ft. dedicated to R&D, process and analytical development, MS&T, and QC.
- **GMP Manufacturing Space:** ~11,000 sq. ft. for multi-product clinical manufacturing with 2X expansion capability.
- **Manufacturing Flexibility:** From small-scale to toxicology-scale material with **streamlined tech transfer to IND in <15 months.**
- **Supply Chain Resilience:** **Dual-source strategy** for critical materials ensuring uninterrupted supply.
- **Fully GMP-compliant warehouse:** Enables **end-to-end material control and traceability** through NetSuite.
- **Sustainability Focus:** Circular practices emphasizing **reuse, in-house builds, and recycling**, to enhance efficiency.

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

~100,000 ft²
facility in Cranbury, NJ



Danon Disease is a Highly Aggressive Rare Genetic Cardiomyopathy

Danon Disease



Disease Etiology

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



Therapeutic Challenges

Standard of care

- Heart transplant

Limitations

- Considerable morbidity and mortality
- Only **~20%** of patients receive HTx¹
- Not curative of extracardiac disease

Clinical Manifestations



Impaired autophagy

- Prominent autophagic vacuoles
- Myocardial disarray



Severe cardiomyopathy^{1,2}

- Severe left ventricular hypertrophy and life-threatening arrhythmias
- High rate of morbidity due to heart failure, with early death or HTx
- **Males:** Aggressive disease course, median overall survival: 19 years
- **Females:** Delayed median presentation (~20 years later) due to additional X chromosome



Other clinical manifestations

- Skeletal myopathy
- CNS manifestations
- Ophthalmologic manifestations

CNS, central nervous system; LAMP2, lysosome-associated membrane protein 2; HTx, heart transplant.

Danon Disease – Cumulative Epidemiology

Hypertrophic Cardiomyopathy (HCM)

- **Anchor Population:** US HCM Prevalence: 700,000 patients^{1,2}
- **Age-Adjustment:** Age distribution of prevalent HCM patients likely older relative to DD patients
- **Weighted, Age-Adjusted Average LAMP2 Prevalence Rate:** 1.85% of HCM patients consistently identified with LAMP2 mutations in multiple studies with >1,000 subjects evaluated³

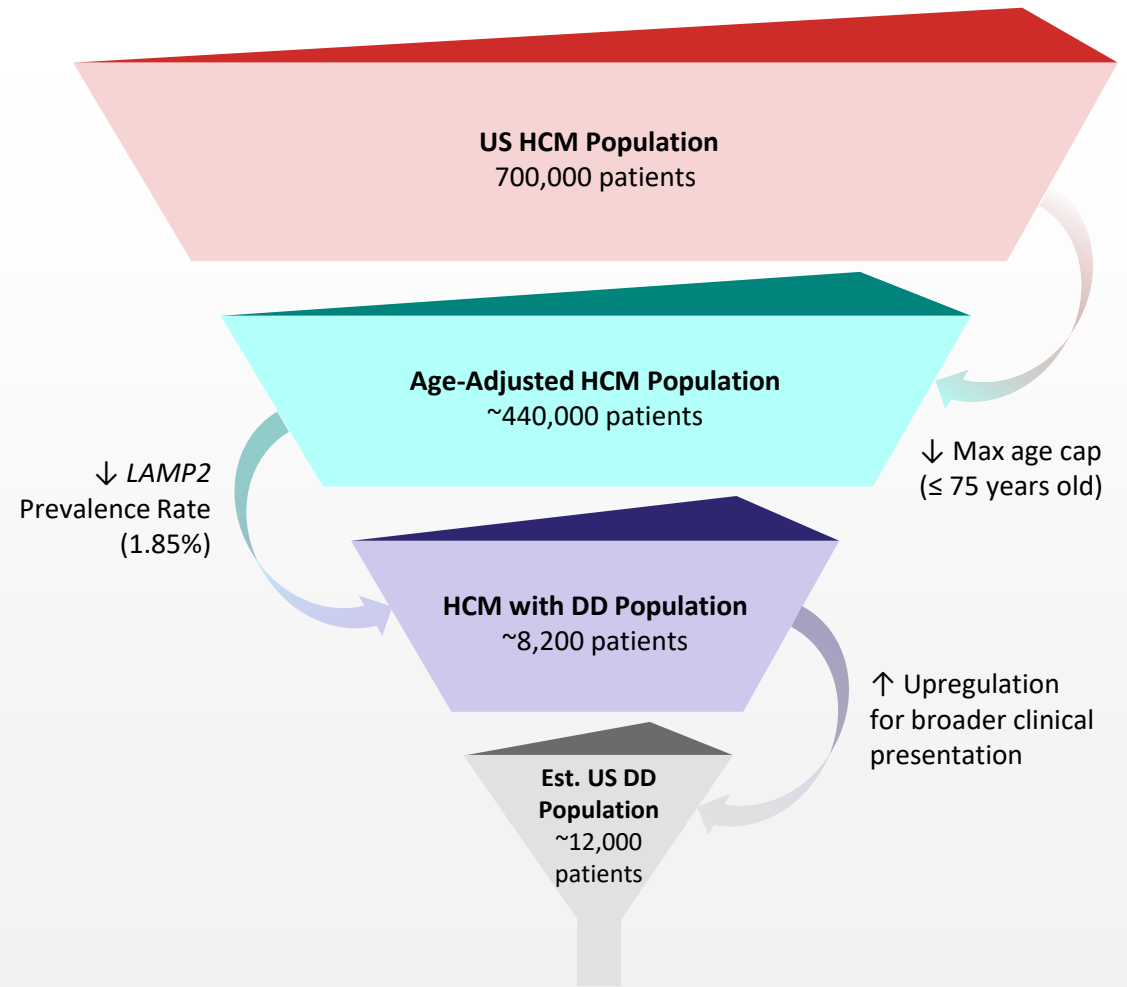
Non-HCM Presenting Danon disease

- Not all DD patients will present with HCM. Reliance on a model based solely on HCM underestimates the true disease burden, particularly in women and in patients presenting with other cardiac features
- **Non-HCM diagnosis Upregulation:** A multiplier is applied to account for male and female DD patients without an HCM diagnosis code³
 - ~30% of DD males
 - ~50% of DD females

Market Opportunity – US and EU

Prevalence of **15,000 to 30,000** individuals

Annual incidence of **800 to 1,200** individuals



DD, Danon disease; HCM, hypertrophic cardiomyopathy, LAMP2, lysosomal associated membrane protein 2

RP-A501 Phase 1 Study: Sustained LAMP2 Expression in Cardiomyocytes

Durable myocardial LAMP2 protein expression seen in all patients

Myocardial LAMP2 Protein Expression

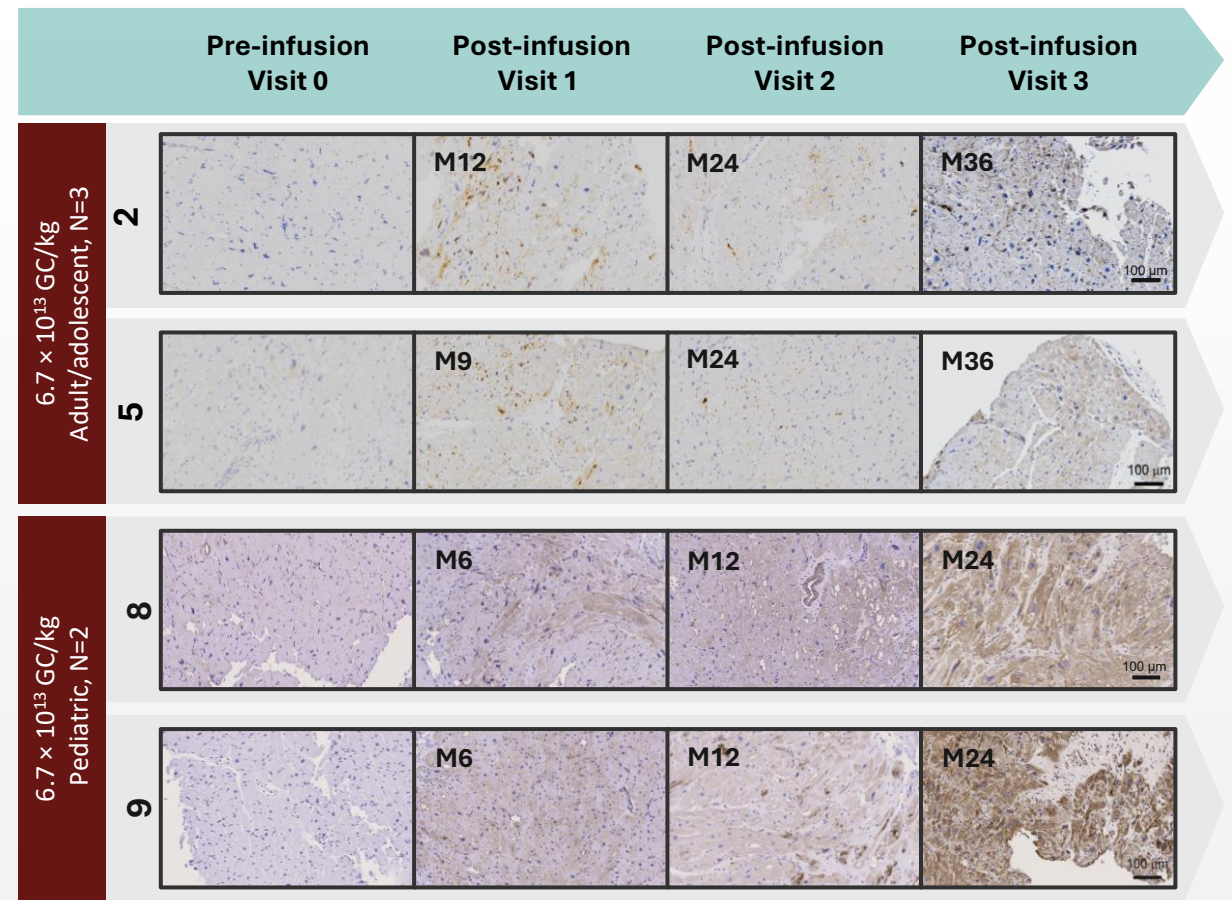
Cohort	Patient	BL	M6	M12	M18	M24	M30	M36	M60
6.7 × 10 ¹³ GC/kg Adult/adolescent	1	0	++	+	NP	NP	0*	0*	+++ [‡]
	2	0	NP	+++	++	++	++	++	
	3	0	NP	+++ [†]	NP	+	+	+	
1.1 × 10 ¹⁴ GC/kg Adult/adolescent	4	0	+	+	+	+	NP	++	
6.7 × 10 ¹³ GC/kg Pediatric ^a	6	0	++	+	NP	++			Analysis Pending
	7	0	+	++	NP	++			

[†]Reflects P 5 9M visit biopsy as 12M biopsy not performed
[‡]Preliminary assessment of biopsy from Patient 1 Y5 visit with updated IHC assay

Legend: IHC Staining Grade[¶] (% Positive Cardiomyocytes)

Grade 0 = no staining	+	= Grade 1 (≤25%)	+++	= Grade 3 (51%–74%)
NP = not performed	++	= Grade 2 (26%–50%)	++++	= Grade 4 (≥75%)

Representative LAMP2 IHC Images



BL, baseline; IHC, Immunohistochemistry; LAMP2, lysosome-associated membrane protein 2; m, month(s); y, year(s)
 Data up to month 36 timepoint adapted from Greenberg B., et al. *N Engl J Med.* 2025;392(10):972-983; Month 60 data for patient 1001 referenced from Data on file

^aPediatric patients received 4.1-4.3x10¹³ GC/kg dose based on a weight and age-based total viral particle (TVP) cap adjustment implemented for pediatric patients

*Patient 1 demonstrated Grade 0 LAMP2 protein IHC staining at the 30- and 36- month assessments, however, Patient 1's LAMP2B vector RNA and DNA (VCN) levels have persisted through 36 months of follow-up

[¶]Grading of LAMP2 protein expression by IHC was done by a board-certified pathologist in a blinded fashion. The semi-quantitative grading reflects the extent of LAMP2 protein expressing cardiomyocytes in the entirety of biopsy sample according to the scale

RP-A501 Phase 1 Study: Benefit Observed Across All Key Parameters

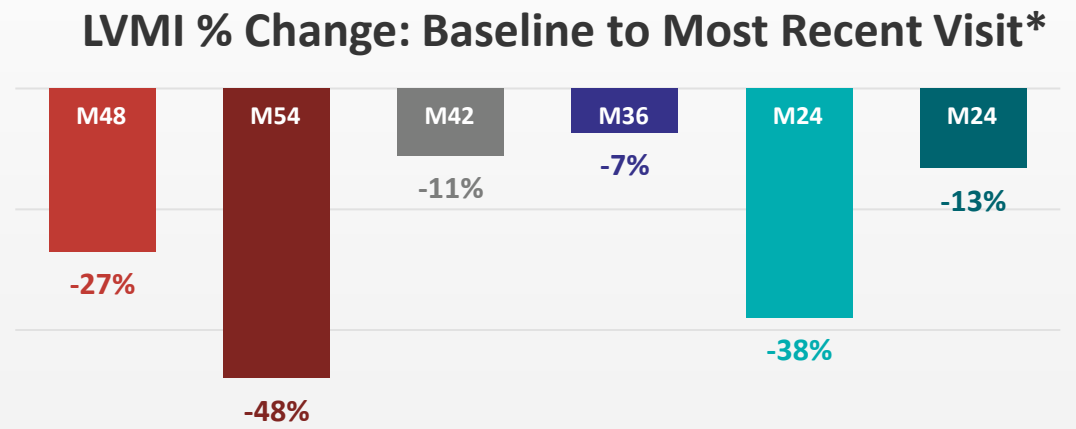
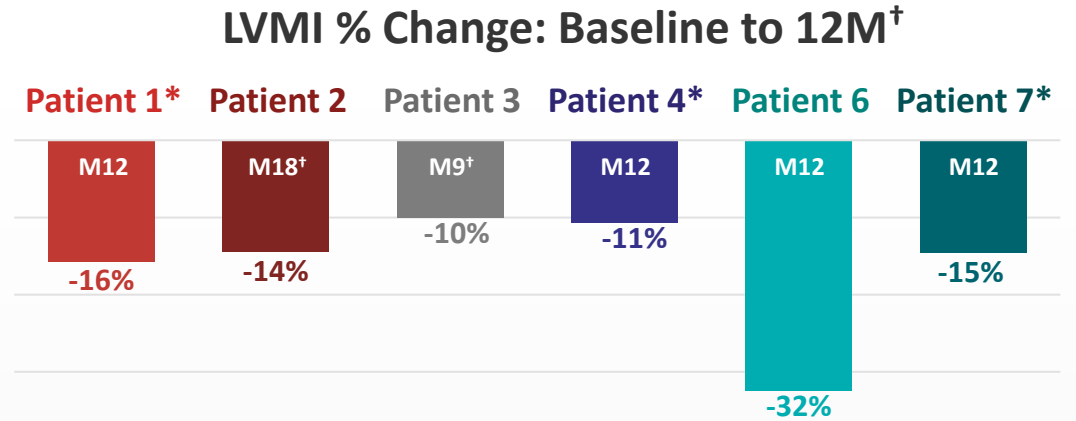
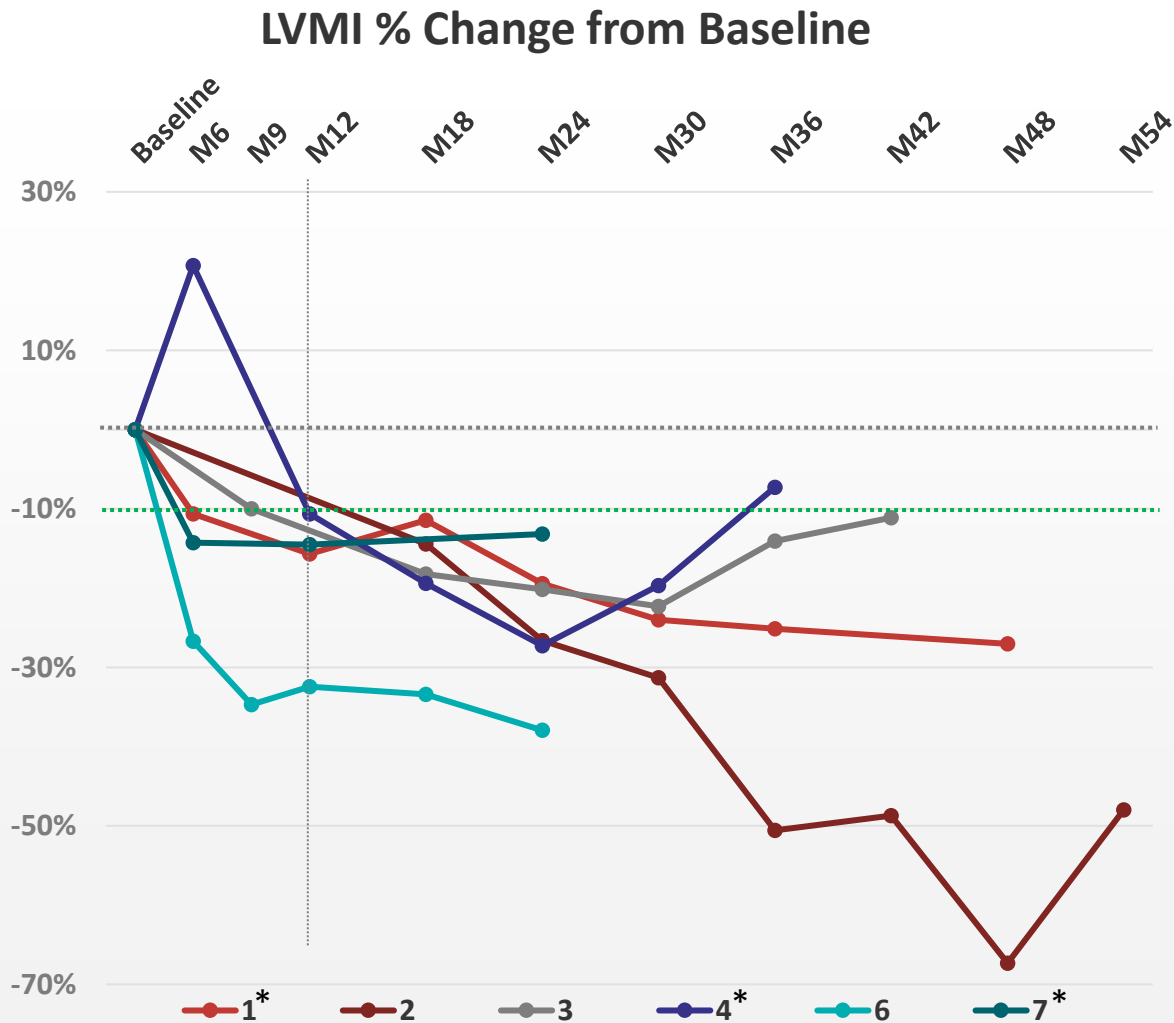
Early LAMP2, BNP, cTnI changes associated with sustained clinical improvement and guided Phase 2 endpoint selection

Cohort	Patient	Age at Most RV (y)	Most Recent Visit (m)	LVEF BL to RV (%)	Δ LVMI,* BL to RV (g/m ^{2.7})	Δ IVSd, BL to RV (mm)	Δ LVPWd, BL to RV (mm)	Δ NT-proBNP, BL to RV (ng/L)	Δ cTnI,† BL to RV (ng/mL)	Δ NYHA Class	Δ KCCQ-12 OS, BL → RV
1:Low Dose Adult/ Adolescent	1	22.3	54	57 to 64	-33%, 85 to 56.9	-6%, 19.8 to 18.6	-20%, 18.8 to 15	-17%, 336 to 279	-99% 0.6 to 0.01	II to I	+52, 44 to 96
	2	24.9	54	55 to 66	-48%, 260.2 to 135.3	-52%, 60.1 to 28.6	-49%, 39.1 to 19.8	-93%, 5119 to 351	-96%, 1.46 to 0.06	II to I	+27, 64 to 91
	3	21.8	42	65 to 59	-11%, 98.2 to 87.3	-10%, 30.9 to 27.8	-27%, 32.1 to 23.4	+16%, 841 to 975	-33%, 0.28 to 0.19	II to I	+7, 77 to 84
2:High Dose Adult/ Adolescent	4	23.9	36	62 to 51	-7%, 68.6 to 63.6	+5%, 18.0 to 19.0	-27%, 24.0 to 17.4	-65%, 720 to 249	-39%, 0.47 to 0.29	II to I	+9, 79 to 89
3:Low Dose Pediatric	6	14.4	24	74 to 78	-38%, 141.5 to 87.8	-19%, 42.4 to 34.2	+1%, 22.8 to 23.1	-78%, 1629 [‡] to 360 [‡]	-85%, 1.78 to 0.27	II to I	+27, 50 to 77
	7	13.7	24	77 to 77	-13%, 82.0 to 71.2	+12%, 18.5 to 20.8	-3%, 14.9 to 14.4	-48%, 1912 to 998	-82%, 1.08 to 0.20	II to I	+30, 52 to 82

* Centrally evaluated (blinded) MRI data were utilized for LVMI when available. All other measurements of cardiac structure and function reflect centrally evaluated (blinded) echocardiogram data.

† Central laboratory assessment of cTnI were performed on cryopreserved and non-cryopreserved samples. Values for cTnI from high-sensitivity and earlier tests. high-sensitivity and earlier assay are expressed in ng/mL. ■ Improved ■ Stabilized ■ Worsened

RP-A501 Phase 1 Study: Sustained Improvements in LV Mass Index



All patients showed ≥10% LVMI decrease at ~12M; improved or sustained at most recent visit

LVMI, left ventricular mass index; MRI, magnetic resonance imaging; m, month(s)

Data published in Greenberg B., et al. *N Engl J Med.* 2025;392(10):972-983 [supplementary appendix]; Data cut-off: April 19, 2024

*Where possible, cardiac MRI assessments shown (Patient 1, 6, and 9); otherwise, echocardiogram data presented. All assessments were conducted by a single reviewer blinded to both patient and timepoint, except for Patient 1 cardiac MRI data, which includes reads from multiple reviewers (note, these data not included in *NEJM* publication). Patient 1 most recent visit with MRI assessment was at 48m

[†]Utilized 9m or 18m data when 12m assessment was not done

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)**

Primary Endpoint Reasonably Likely to Predict Clinical Benefit

Justification for use of LAMP2 protein expression and LV Mass

Support for Co-Primary Endpoints

- 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

WT Full Length LAMP2 Protein Expression

- **Epidemiologic support:** Mutation of *LAMP2* is the root cause of Danon disease and even modest levels of LAMP2 confer a 2-decade survival advantage in female patients
- **Pre-clinical support:** LAMP2 restoration conferred histologic, functional and survival benefits in *LAMP2* knock-out model¹
- **Phase 1 trial support:** LAMP2 expression associated with decreased vacuolar area, improved myofibrillar disarray, clinical improvement

Left Ventricular Mass

- **Pathology support:** Severity of the cardiomyopathy in Danon disease is the major prognostic factor and severe left ventricular hypertrophy is the most common manifestation
- **Natural history support:** Retrospective natural history shows year-over-year increases in LV mass in Danon disease patients
- **Phase 1 trial support:** Consistent and significant reductions in LV mass as early as 6 months by echocardiography and cardiac MRI

Phase 2 Trial Design of RP-A501 in Male Patients with DD

Pivotal, global, single-arm, open-label trial¹



Key eligibility criteria

Males age ≥8 years, *LAMP2* mutation, NYHA II-III, evidence of LV hypertrophy, elevated hsTnl



Co-Primary Endpoint

To support accelerated approval, co-primary endpoint consisting of improvements in *LAMP2* protein expression (≥ Grade 1) and reductions in Left Ventricular Mass (LVMI; ≥10% ↓) at 12-month post-infusion



Current Status

Preparations for Cohort 4 dosing is ongoing; treatment anticipated in the first half of 2026

Cohort 1

Adult/Adolescent (n=1)

6.7 x 10¹³ GC/kg of RP-A501

IM regimen: Rituximab + Steroid + Sirolimus



Patient 1

Cohort 2

Pediatric (n=3)⁺

6.7 x 10¹³ GC/kg of RP-A501

IM regimen: Rituximab + Steroid + Sirolimus

**Including a 90-day Pediatric Safety Run-in (n=2)*



Patient 2



Patient 3



Patient 4

Cohort 3

Adult/Adolescent and Pediatric with C3 inhibitor (n=2)

6.7 x 10¹³ GC/kg of RP-A501

IM regimen: Rituximab + Steroid + Sirolimus + C3 inhibitor



Patient 5



Patient 6

Current Stage

Cohort 4

Pediatric and Adult/Adolescent

3.8 x 10¹³ GC/kg of RP-A501

IM regimen: Rituximab + Steroid + Sirolimus

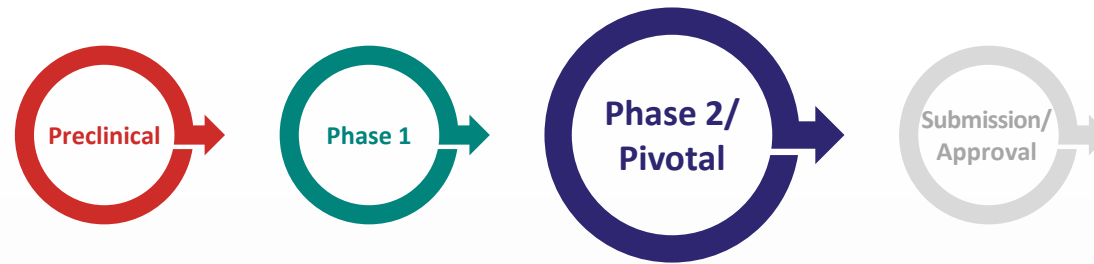
*Staggered dosing of initial 3 patients

Next Steps

Following the treatment of three patients at 3.8x10¹³ GC/kg, we will align with the FDA regarding the completion of the pivotal study

Concurrent Natural History Study²

Pivotal Phase 2 Trial of RP-A501 is Ongoing



Phase 1 Safety & Efficacy

- Phase 1 study of RP-A501 demonstrated durable protein expression, stabilization of cardiac function through 5-years post-infusion and was generally well-tolerated
- All six evaluable Phase 1 patients are alive and transplant-free

Pivotal Phase 2 trial

- Pivotal, global, multicenter, single-arm, open-label Phase 2 clinical study is ongoing
- The study utilizes commercially representative RP-A501 material manufactured at our facility in Cranbury, NJ

NEXT STEPS

- Treatment of Cohort 4

Regulatory Designations

RMAT
and PRIME

Orphan Drug
designation in the US

Rare Pediatric
Disease designation

Fast Track (US),
ATMP

A Cardiomyopathy with High-Risk of Arrhythmias and Sudden Cardiac Death

PKP2-Arrhythmogenic Cardiomyopathy (PKP2-ACM)



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

Standard of care

- Beta-blockers, anti-arrhythmic agents, ICD, and ablation.

Limitations

- Available treatments do not modify disease progression; no curative therapeutic options
- ICD firings are lifesaving but highly traumatic events

Market Opportunity¹ – US and EU

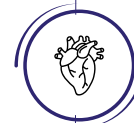
Prevalence of **50,000** individuals

Clinical Manifestations



Susceptibility²

- Frequent and/or endurance exercise are associated with increased likelihood of ACM diagnosis and severity
- Patients are often advised to avoid strenuous activity



Associated Risks³

- Mean age at presentation: 35y (±18)
- Disease progression may lead to heart failure, premature death, or transplant



In one study population of 439 ARVD/ARVC patients: ⁴ (median follow up, 7 years)

- >70%** experienced sustained ventricular arrhythmias
- >80%** received ICD placement
- ~11%** of patients experienced SCD or resuscitated SCD

PKP2-ACM Prevalence in the US and EU

ACM prevalence

1:1000 to 1:5000^{1,2}

PKP2 variants

32.9%

2,572 ACM patients assessed from 13 publications an aggregated mean of **32.9% had PKP2 mutations**³

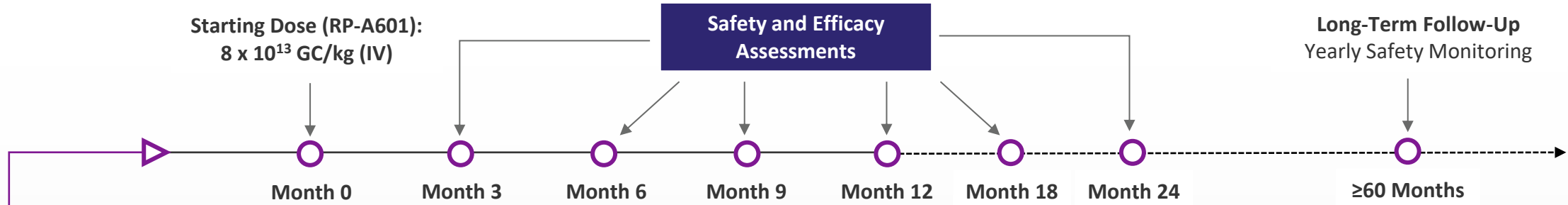
ACM-PKP2 US & EU Prevalence

~50,000

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

Phase 1 Trial Design of RP-A601 in Adult Patients with PKP2-ACM

First-in-human, multi-center, open-label, dose escalation trial¹



INCLUSION CRITERIA

- Male or female ≥18 years
- Clinical diagnosis of ACM as defined by the 2010 revised Task Force Criteria
- Pathogenic or likely pathogenic truncating variant in *PKP2*
- Anti-AAVrh.74 capsid neutralizing antibody assay ≤1:40
- History of ICD implantation ≥6 months prior to enrollment

EXCLUSION CRITERIA

- Cardiomyopathy related to a genetic etiology other than *PKP2* truncating variant
- Previous participation in a study of gene transfer or gene editing
- Severe right ventricular dysfunction
- Left ventricular ejection fraction by echocardiogram ≤50%
- New York Heart Association Class IV heart failure

ENDPOINTS

Primary Endpoints: Safety

- Incidence of TEAEs and SAEs
- Identification of dose limiting toxicities

Secondary & Exploratory Endpoints: Efficacy

- Change in PKP2 protein expression
- Change in frequency of clinical markers of life-threatening ventricular arrhythmias
- Cardiac biomarkers

Natural history study² planned to provide context for the Phase 1 trial and additional information on disease progression

Preliminary Indications of Improvement or Stabilization with RP-A601

in Clinical Markers of Arrhythmia Burden, Heart Function and QoL

Patient	Age at Enrollment (y)	Most Recent Visit (MRV, m)	Heart Function & QoL			Arrhythmia Burden		
			RV Systolic Function (ECHO) BL → MRV	KCCQ-12 Score BL → MRV	NYHA Class BL* → MRV	PVCs per 24h BL → MRV	NSVTs per 24h BL → MRV	T-wave inversions (precordial and inferior ECG)
1	55	12	Normal → Normal [†]	+87% 39.6 → 74.0	II → I	-63% 117** → 43	0 → 0	0 → 0
2	58	9	Mild - Moderate Reduced → Normal	+76% 54.2 → 95.3	II → I	-9% 2974 → 2713	0 → 0	4 → 4
3	36	6	Normal → Normal	0% 59.4 → 59.4	II → II	-60% 2650** → 1057	5** → 0	6 → 2

*As assessed on Day-14 pre-infusion.

**Ambulatory rhythm monitoring was conducted at baseline for 48 hours. For the specified patients, data from two separate 48-hour rhythm monitoring studies were combined to produce the baseline value. Rhythm monitoring at the most recent visit was conducted for 7 days.

[†]Stability of RVEF was corroborated by month 12 cardiac magnetic resonance (CMR) s imaging.

Ongoing FDA Engagement for RP-A601 in PKP2-ACM



Phase 1 Safety

- RP-A601 was generally well-tolerated with no dose-limiting toxicities observed
- No TMA or ventricular arrhythmias were observed

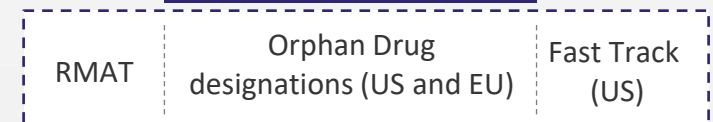
Phase 1 Efficacy

- **All 3 patients demonstrated cardiac transduction** with localized myocardial protein expression (PKP2, Desmocollin-2, Cadherin-2) that was maintained or increased up to 12-months post-infusion
- **Preliminary indications of improvement or stabilization observed** in arrhythmia burden, heart function, and quality of life with up to 12-months of follow-up
 - **Decreased/stabilized ventricular ectopy (PVC, NSVT)** on rhythm monitoring in all patients
 - **Decreased/stabilized T-wave inversions** on ECG in all patients
 - **Improved/stabilized RV function**
 - **Improved QoL and NYHA Class** in patients followed beyond 6 month

NEXT STEPS

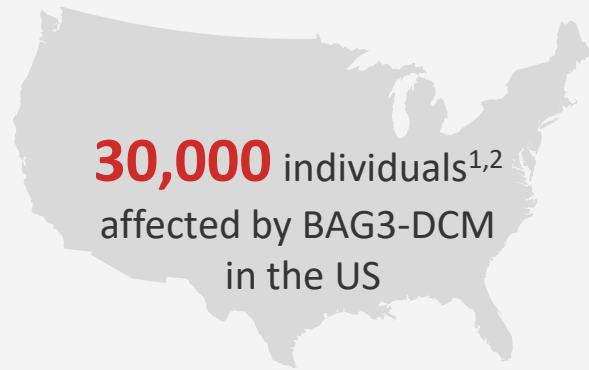
- With no further dose escalation plans, Rocket is engaging with FDA on a potential pivotal trial design to evaluate the efficacy and safety of RP-A601

Regulatory Designations



Cardiovascular Health is Dependent on Functional BAG3 Protein

BAG3-Dilated Cardiomyopathy (BAG3-DCM)

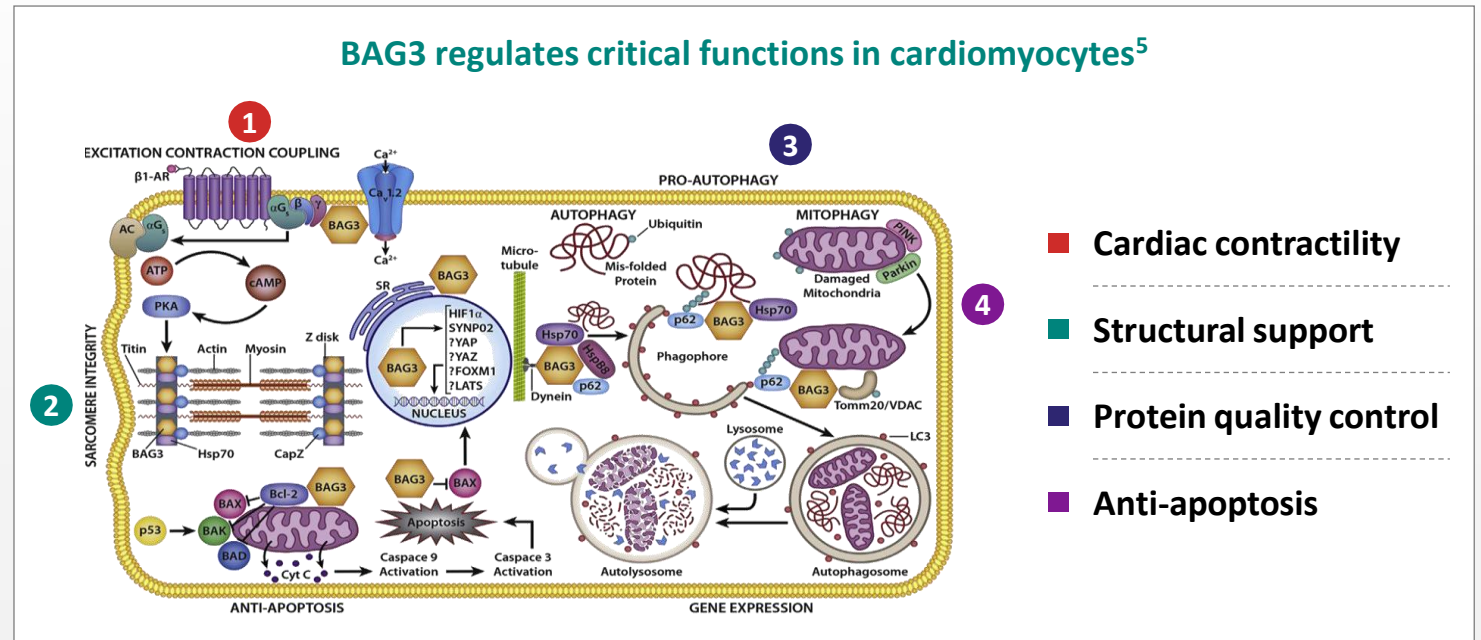


30,000 individuals^{1,2} affected by BAG3-DCM in the US

- **20%-50%** of DCM patients have familial DCM; **up to 40%** of whom have an identifiable genetic cause^{3,4}
- **80%** of patients over 40 years of age with a BAG3 mutation will present with the disease²
- Pathogenic variants in BAG3 are estimated to cause **2.3%-6.7%** of DCM cases in the US, Europe, & Japan²

Disease Etiology

- Autosomal dominant mutations in *BAG3* gene
- Loss of *BAG3* leads to an accumulation of misfolded and damaged proteins, which impairs the heart's ability to contract, leading to heart failure



BAG3-DCM Patients are at High Risk of Progression to End Stage Disease

Clinical Manifestations



Dilated cardiomyopathy (DCM)

- Characterized by progressive thinning of the walls of the heart, causing dysfunction of left ventricular contractility
- Impairment of the heart’s ability to contract, leads to impaired cardiac function, heart failure, and even premature death



Other Associated Risks

- Patients have significant limitations in activities of daily living (e.g., employment, walking, personal care)

Therapeutic Challenges



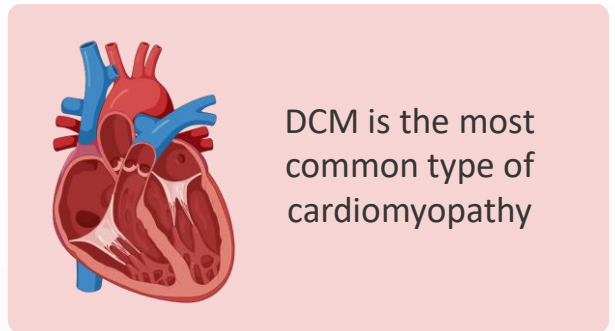
Standard of care

- Medical therapies for heart failure and/or interventional therapies (ICD, cardiac resynchronization, and heart transplant)
- SOC therapies demonstrate limited efficacy, with only 2.9% of BAG3-DCM patients experiencing normalization of LVEF during follow-up¹



Limitations

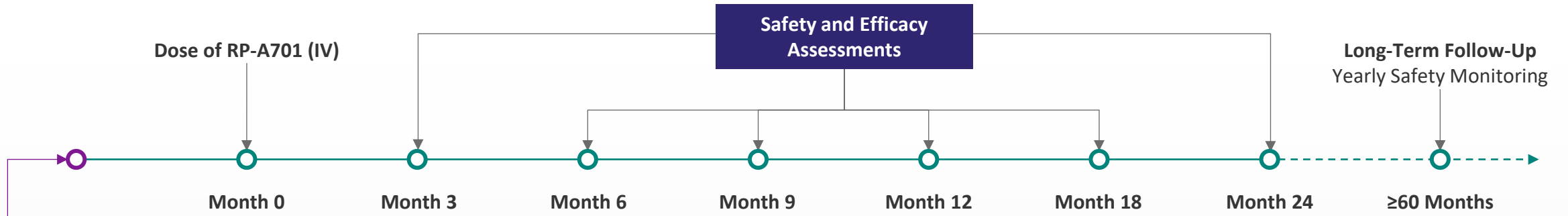
- Available treatments do not address the underlying cause of disease
- Heart transplantation is the only definitive therapy, but carries short- and long-term risks, including post-transplant mortality, and limited by availability



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

Phase 1 Trial Design of RP-A701 in Adult Patients with BAG3-DCM

First-in-human, open-label, dose escalation trial¹



INCLUSION CRITERIA	EXCLUSION CRITERIA	ENDPOINTS
<ul style="list-style-type: none"> • Individuals between 18-65 years with a clinical diagnosis of DCM • Pathogenic or likely pathogenic variant in BAG3 • History of ICD implantation ≥ 3 months prior to enrollment • NYHA Class II or III HF symptoms with stable HF therapeutic guideline-directed medical regimen for ≥ 30 days enrollment 	<ul style="list-style-type: none"> • Cardiomyopathy related to an etiology other than BAG3 mutations • Previous participation in a study of gene transfer or gene editing • I.V. inotropic, vasodilator, or diuretic therapy ≤ 30 days prior to enrollment • Severe right ventricular dysfunction • Left ventricular ejection fraction by echocardiogram $< 25\%$ 	<p>Primary Endpoints: Safety</p> <ul style="list-style-type: none"> • Incidence of TEAEs • Incidence of SAEs • Identification of dose limiting toxicities <p>Secondary & Exploratory Endpoints: Efficacy</p> <ul style="list-style-type: none"> • Change in BAG3 protein expression • Change in clinical markers of cardiovascular function and heart failure • Quality of life

Current Status: Phase 1 trial start-up activities are underway for RP-A701, and dosing of the first patient is anticipated in mid-2026.



Severe LAD-I: A Life-Threatening Pediatric Genetic Immune Disorder

Severe Leukocyte Adhesion Deficiency-I (LAD-I)



Disease Etiology

- **ITGB2** gene mutations (21q22.3), encoding the beta-2-integrin, CD18
- CD18 absent, dysfunctional, or reduced on neutrophils; essential for leukocyte adhesion to endothelium and expressed along CD11 integrins
- ~65% of LAD-I patients are classified as severe^{1,2}.



Therapeutic Challenges

Standard of care

- Allogeneic HSCT or supportive care

Limitations

- Donor availability
- Frequent GvHD
- Infections
- Graft failure

Clinical Manifestations



Patients suffer from severe, recurrent infections; fatal in majority^{1,2}

- An ultra-rare genetic immune disorder that affects white blood cells' ability to reach sites of infections or injury to fight off pathogens, causing recurrent life-threatening bacterial and fungal infections.
- Ultimately, the condition prevents a patient's immune system from adequately responding to infection and injury, predisposing patients to recurrent and often fatal infections.



Associated Risks

- Severe LAD-I patients experience frequent severe infection despite prophylactic antibiotics (**average 3-8 per year⁴**)
- Without transplant, **greater than 60%^{1,2,5}** of children with severe LAD-I die before two years of age, **less than 15%⁵** surviving beyond the age of 9
- In absence of definitive therapy, all children with severe LAD-I continue to be at high risk of contracting, life-threatening severe infections

Market Opportunity¹⁻³ – US

Prevalence of
1:1 million

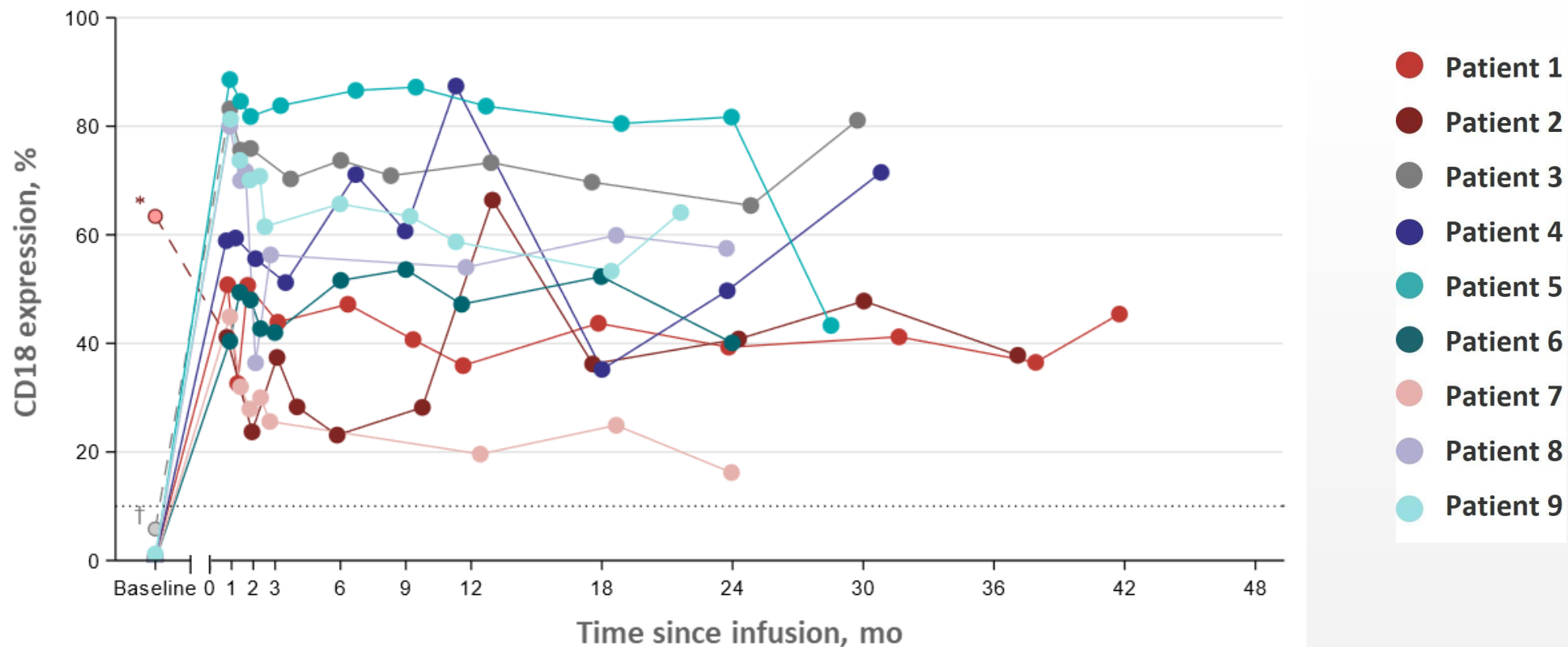
Annual incidence of
~25 individuals

Allo, allogeneic; GvHD, graft-versus-host disease; HSCT, hematopoietic stem cell transplant; *ITGB2*, integrin subunit beta 2; LAD-I, leukocyte adhesion deficiency-I.

1. Fazlollahi et al. *Pediatr Allergy Immunol.* 2023.; 2. Almarza Novoa et al. *J Allergy Clin Immunol Pract.* 2018.; 3. Moutsopoulos et al. *PLoS Pathog.* 2015.; 4. Kambli et al. *Front Immunol.*, 2020.; 5. Madkaikar et al., *Indian Pediatr.*, 2012.;

CD18 Expression in PB Polymorphonuclear Cells in Pivotal Trial

Sustained >10% PMN CD18 expression 1 year after infusion across the entire cohort

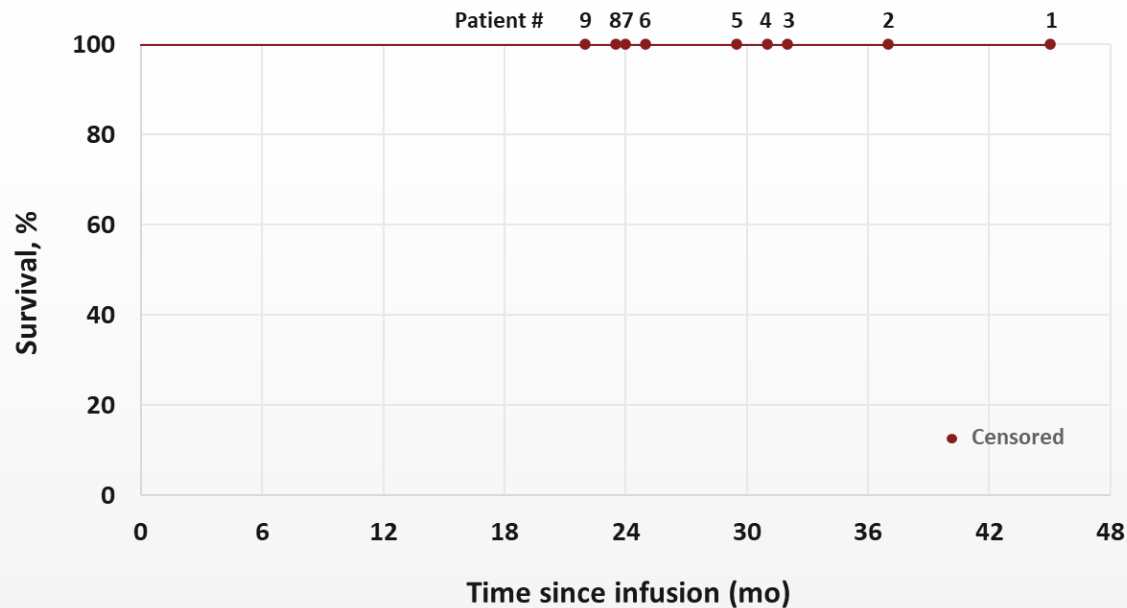


*Baseline dim or weak CD18 neutrophil expression in Patient 2 in 63.4% of cells with CD11a or CD11b neutrophil expression of less than 2% most likely indicates abnormal or unstable protein.

†Baseline CD18 neutrophil expression in Patient 3 in 5.8% of cells with CD11a or CD11b neutrophil expression of less than 2% most likely indicates abnormal or unstable protein.

100% HSCT-free Survival in Pivotal Trial and Reduction in Hospitalization

100% HSCT-free survival Kaplan–Meier estimate

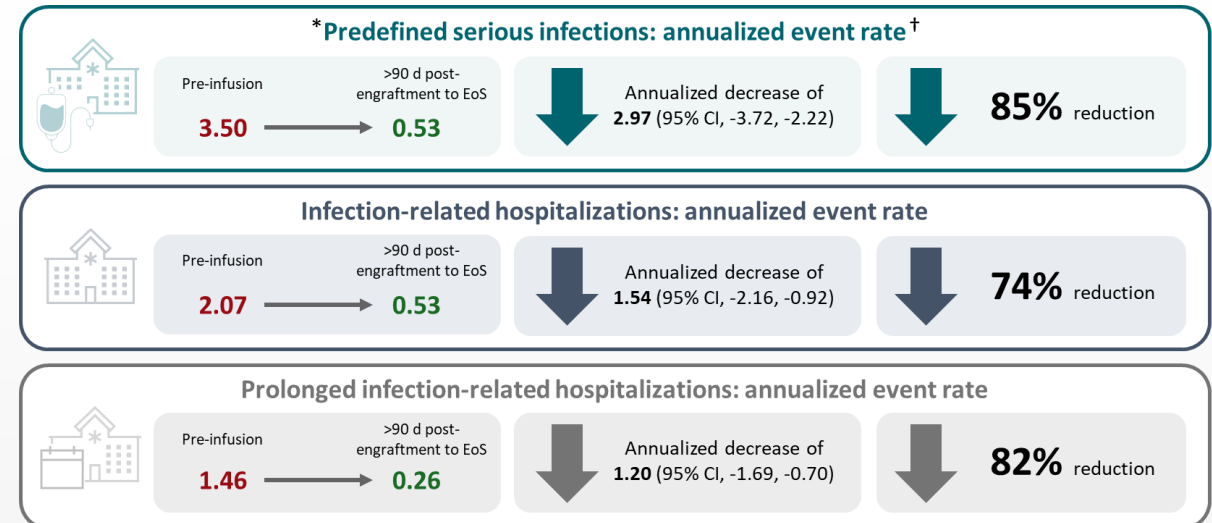


100% (9/9) treated patients achieved

HSCT-free survival (survival without allogeneic HSCT)

- at least 1 year post–RP-L201 infusion AND
- at two years of age among patients who were younger than one year of age at enrollment.

Meaningful reduction in infection-related hospitalizations following immune reconstitution

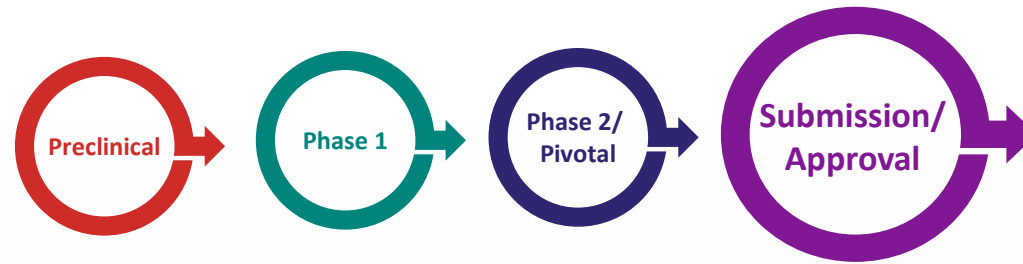


* Predefined serious infections were those infections requiring hospitalization or parenteral (intravenous) antimicrobials.

[†] Annualized event rate is calculated as the Total Number of Events / Total Time. Results are adjusted event rate per year. Pre-infusion includes all lifelong medical history prior to RP-L201 infusion.

- *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)*

KRESLADI™ (RP-L201) for Severe LAD-I on Track for March 28, 2026 PDUFA



Initial Efficacy	Top-line Data Readout	NEXT STEPS
<ul style="list-style-type: none"> • 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 	<ul style="list-style-type: none"> • HSCT-free survival for 9/9 patients, ≥ 2 years age and ≥ 1 year post-treatment with 22 to 45 months follow-up • No graft failure or GvHD • No RP-L201 related SAEs 	<ul style="list-style-type: none"> • BLA resubmission was accepted by the FDA in October 2025 • PDUFA date is March 28, 2026 • Rocket is eligible for a PRV, upon its potential approval

Regulatory Designations

RMAT and PRIME

Orphan Drug designation (US and EU)

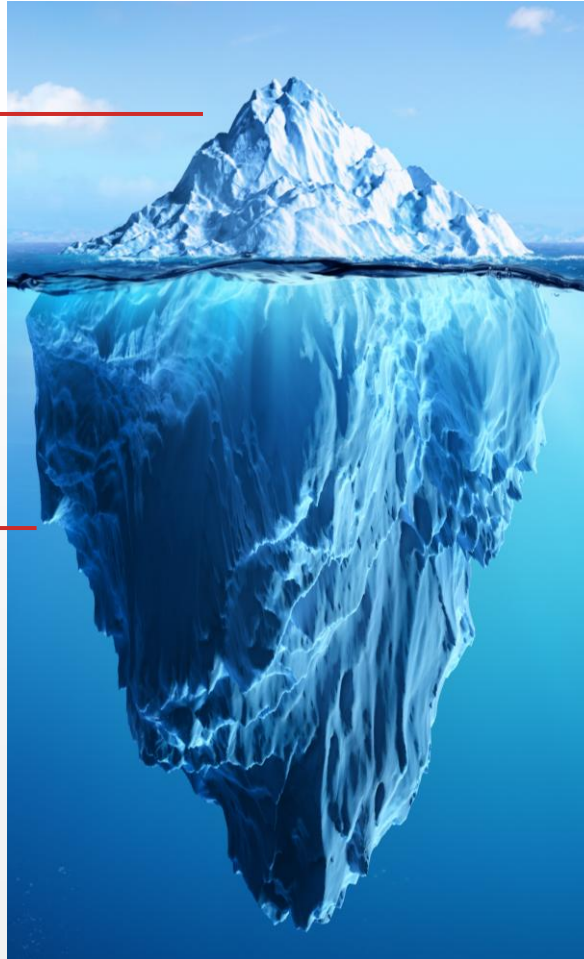
Rare Pediatric Disease designation

Fast Track (US), ATMP

Wave 2: Future Cardiovascular Gene Therapies

Current programs reflect only the tip of the iceberg – Disciplined focus today for scalable innovation tomorrow.

Above the Surface: ○
Current Clinical Pipeline



What Lies Beneath: ○
Future Cardiovascular Opportunities

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

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

Expert Leadership With Proven Track Record



Gaurav Shah, M.D.
Chief Executive Officer

Spearheaded Kymriah development at Novartis towards approval.



Sarbani Chaudhuri
Chief Commercial & Medical Affairs Officer

20+ years of experience driving commercial growth for rare cardiac and hematology launches.



Syed Rizvi, M.D.
Chief Medical Officer

20+ years of experience across all stages of drug development.



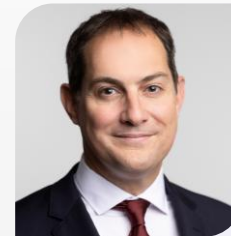
Jonathan Schwartz, M.D.
Chief Science & Gene Therapy Officer

Led multiple biologics approvals.



Chris Stevens
Chief Operating Officer

25 years of proven success in technical operations, product strategy, and leadership.



Martin Wilson, J.D.
General Counsel & Chief Corporate Officer

~20 years legal, compliance and executive experience and accomplishment in life sciences.



Meg E. Dodge, J.D., LL.M.
Senior Vice President, Head of External Affairs

15+ years of experience with capital markets, strategic communications, and corporate operations.





THANK YOU!

