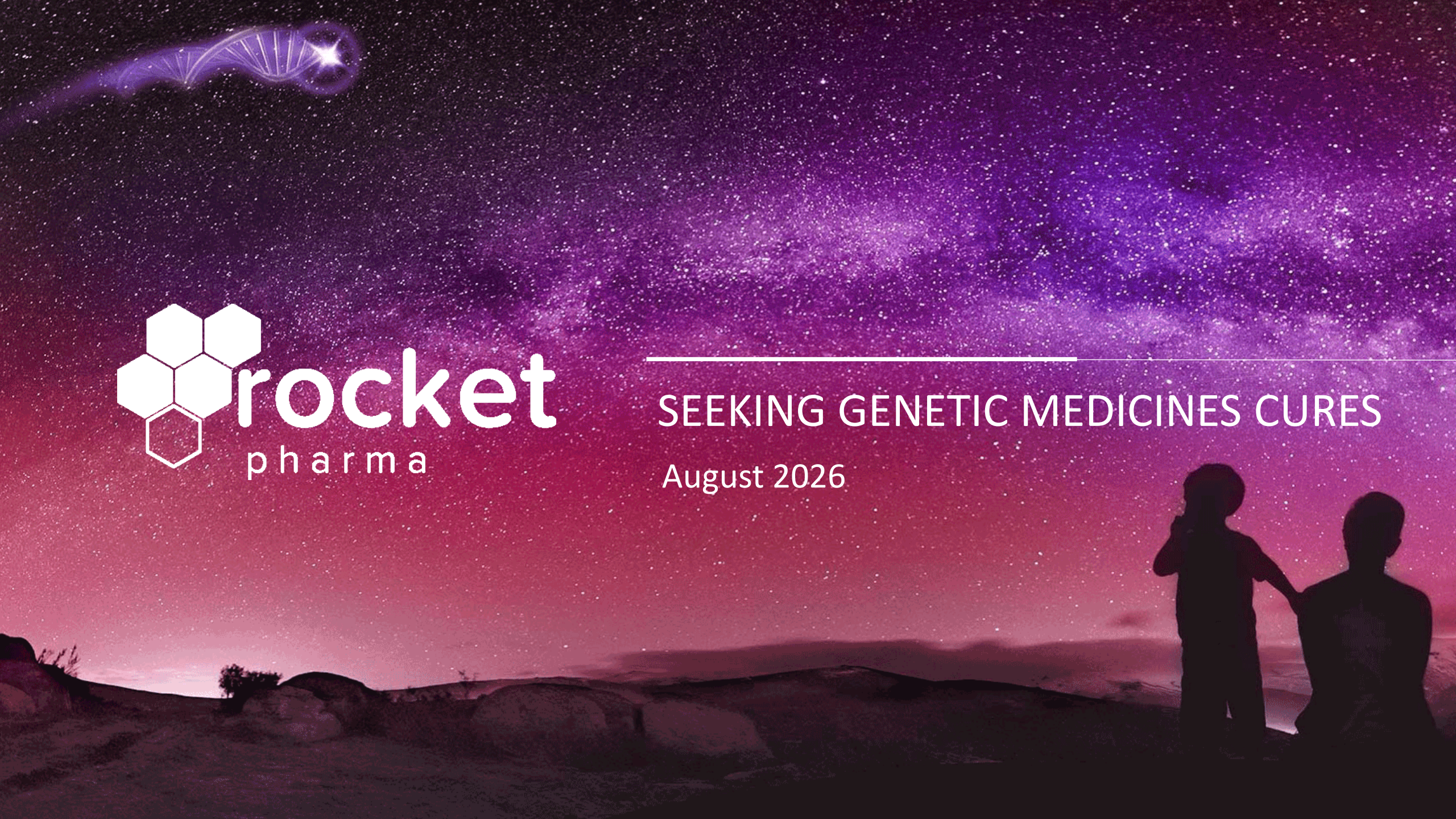


SEEKING GENETIC MEDICINES CURES

August 2026

DISCLAIMER

This presentation contains forward-looking statements concerning Rocket's future expectations, plans and prospects that involve risks and uncertainties, as well as assumptions that, if they do not materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this presentation are forward-looking statements. You should not place reliance on these forward-looking statements, which often include words such as "believe," "expect," "anticipate," "intend," "plan," "estimate," "seek," "will," "may," "suggest" or similar terms, variations of such terms or the negative of those terms. These forward-looking statements include, but are not limited to, statements concerning Rocket's cash runway and financial position, Rocket's planned use of proceeds from the monetization of the KRESLADI™ Priority Review Voucher, Rocket's expectations regarding its ability to obtain additional funding to conduct its planned research and development efforts, the safety and effectiveness of RP-A501 in its pivotal Phase 2 trial under the modified protocol, the Company's ability to align with the FDA on the path to treating additional Danon disease patients, the expected timing and data readouts of Rocket's ongoing and planned clinical trials, the expected timing and outcome of Rocket's regulatory interactions and planned submissions, Rocket's plans for the advancement of its cardiovascular AAV programs and KRESLADI™, including planned pivotal trials, and the safety, effectiveness and timing of related pre-clinical studies and clinical trials, Rocket's ability to establish key collaborations and vendor relationships for its product candidates, Rocket's ability to develop sales and marketing capabilities or enter into agreements with third parties to commercialize its product candidates, and Rocket's ability to expand its pipeline to target additional indications compatible with its gene therapy technologies. Although Rocket believes that the expectations reflected in the forward-looking statements are reasonable, Rocket cannot guarantee such outcomes. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including, without limitation, the results of Rocket's ongoing and planned clinical trials, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, unexpected expenditures, Rocket's competitors' activities, including decisions as to the timing of competing product launches, pricing and discounting, Rocket's ability to develop, acquire and advance product candidates into, enroll a sufficient number of patients into, and successfully complete, clinical studies, Rocket's ability to acquire additional businesses, form strategic alliances or create joint ventures and realize the benefits of such transactions, Rocket's ability to achieve the expected benefits of its portfolio prioritization and strategic restructuring, including extending its cash runway, Rocket's ability to obtain and enforce patents to protect its product candidates, and its ability to successfully defend against unforeseen third-party infringement claims, as well as those risks more fully discussed in the section entitled "Risk Factors" in Rocket's Annual Report on Form 10-K for the year ended December 31, 2025, filed February 26, 2026 with the SEC, and subsequent filings with the SEC, including its 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. This presentation also includes illustrative strategic concepts, implementation frameworks, partnership structures, regulatory strategies and manufacturing scenarios related to potential collaborations in Saudi Arabia. These concepts are preliminary, subject to further evaluation, discussion and agreement among the parties, and should not be interpreted as commitments by Rocket Pharmaceuticals or any other organization. Market opportunity estimates, epidemiology analyses, addressable patient estimates, timelines, revenue illustrations and implementation roadmaps are based on assumptions believed reasonable at the time of preparation but are inherently uncertain and should not be viewed as forecasts or commitments.

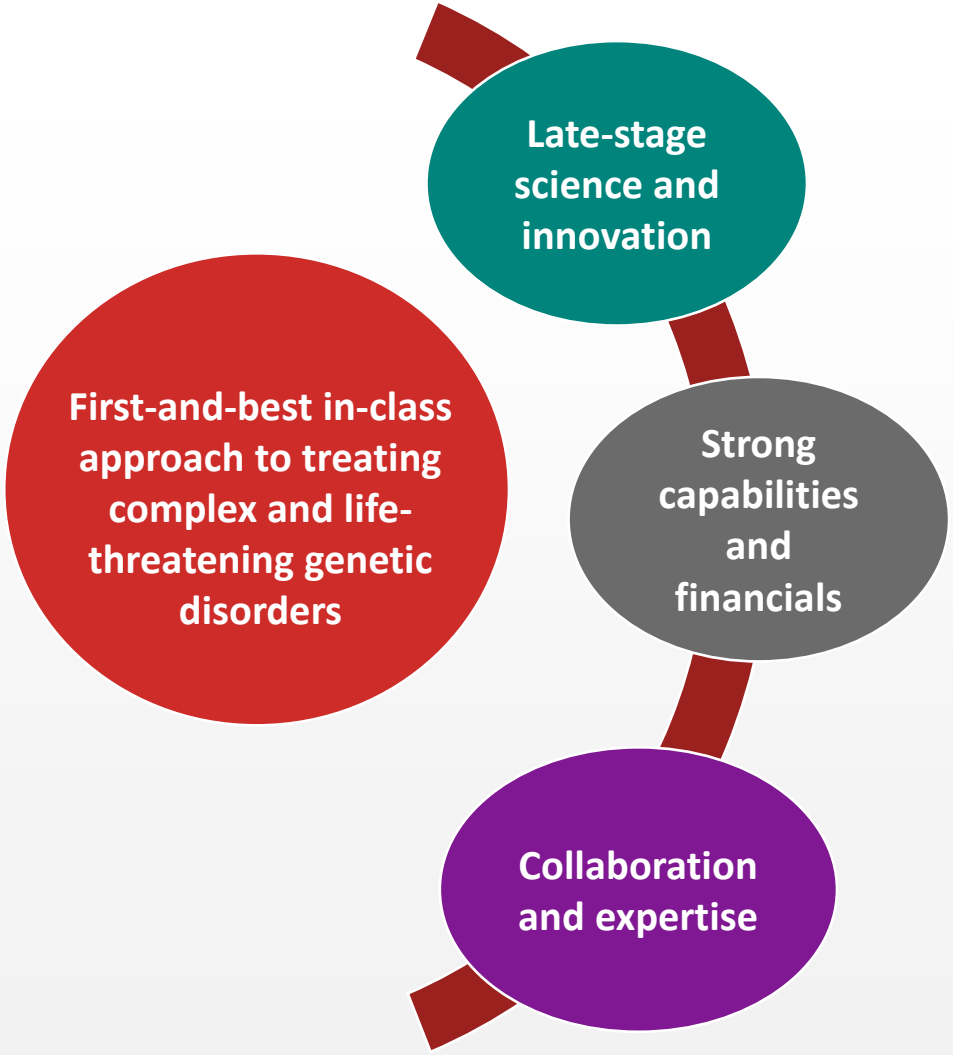
Vision: Seeking Genetic Medicines Cures



Mission

To develop **first-in-class** and **best-in-class curative genetic medicines** for patients with devastating diseases

A Fully Integrated Cardiovascular Rare Disease Company



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

First company with safety and efficacy data for genetic medicines 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

$283.7M[1]

in cash and cash equivalents; **sufficient to fund operations into the second quarter of 2028**

Leadership team with proven track record of

20+

US and ex-US drug approvals and launches

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

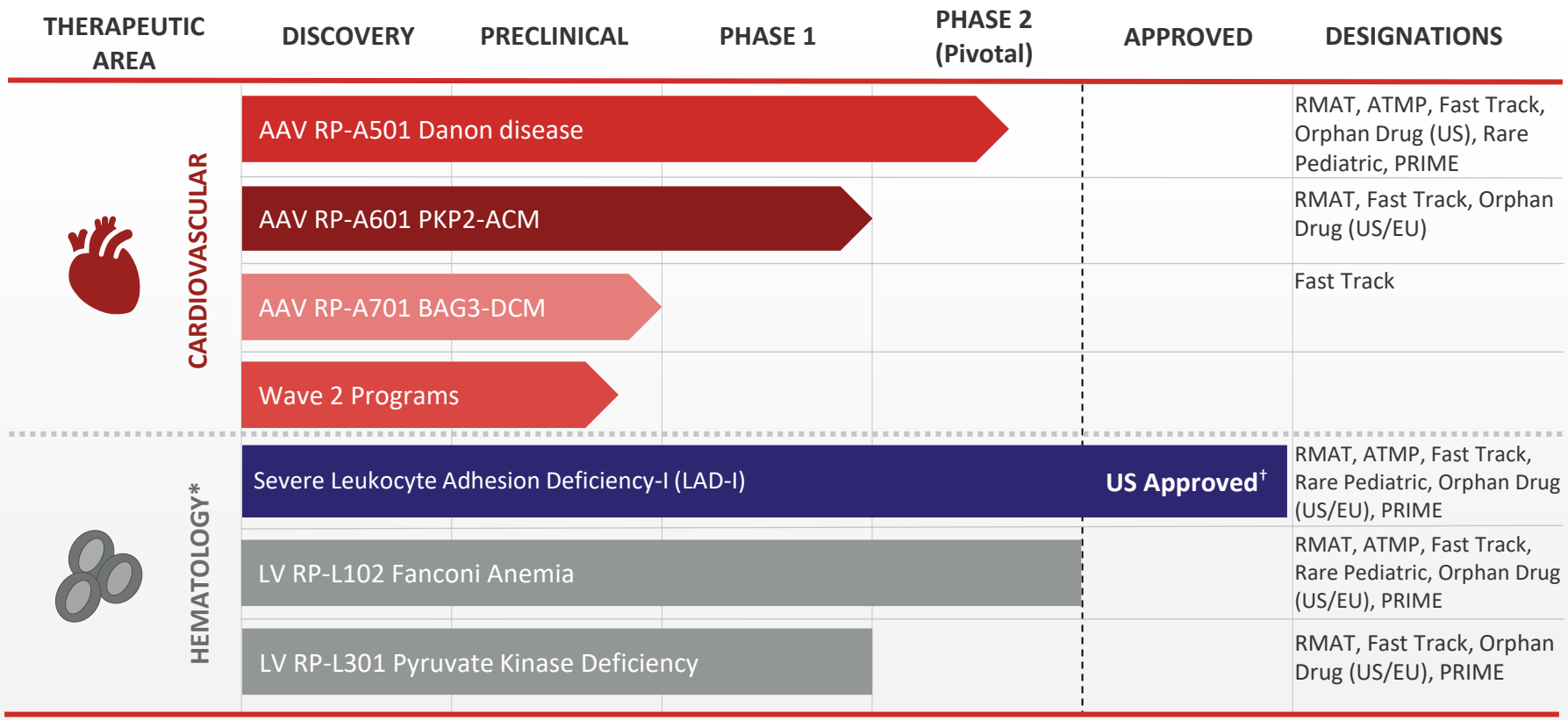
AAV, adeno-associated virus; cGMP, Current Good Manufacturing Practices; HCP, healthcare provider

[1]Cash balance as of June 30, 2026.

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



*Rocket prioritized approval of the severe LAD-I program within its LV portfolio; external partnership opportunities for RP-L102 and RP-L301 are under evaluation.
†Accelerated Approval (AA) granted by FDA on March 26, 2026, for severe LAD-I.

AAV, adeno-associated virus; ATMP, advanced therapy medicinal product; BAG3-DCM, BCL2-associated athanogene 3 dilated cardiomyopathy; LV, lentiviral vector;
MOA, mechanism of action; PDUFA, Prescription Drug User Fee Act; PKP2-ACM, plakophilin 2 related arrhythmogenic cardiomyopathy; PRIME, Priority Medicines; RMAT, regenerative medicine advanced therapy

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

IN VIVO platform

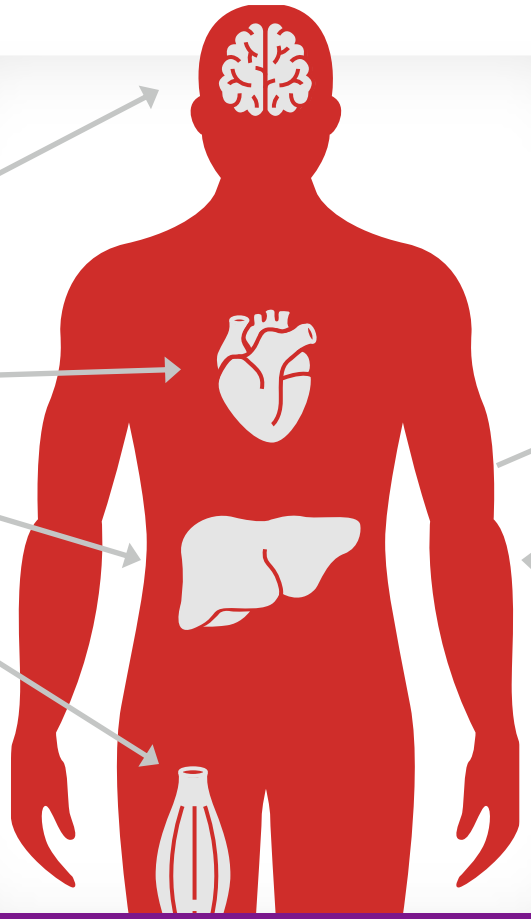
Laboratory-produced AAV

Therapeutic AAV



Direct intravenous injection

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

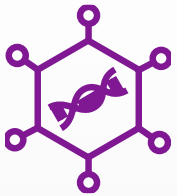


EX VIVO platform

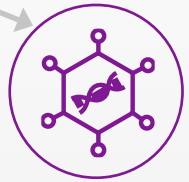
Remove cells and isolate patient HSCs



Laboratory-produced LV



Therapeutic LV



Gene-modified HSCs

Infusion of modified HSCs

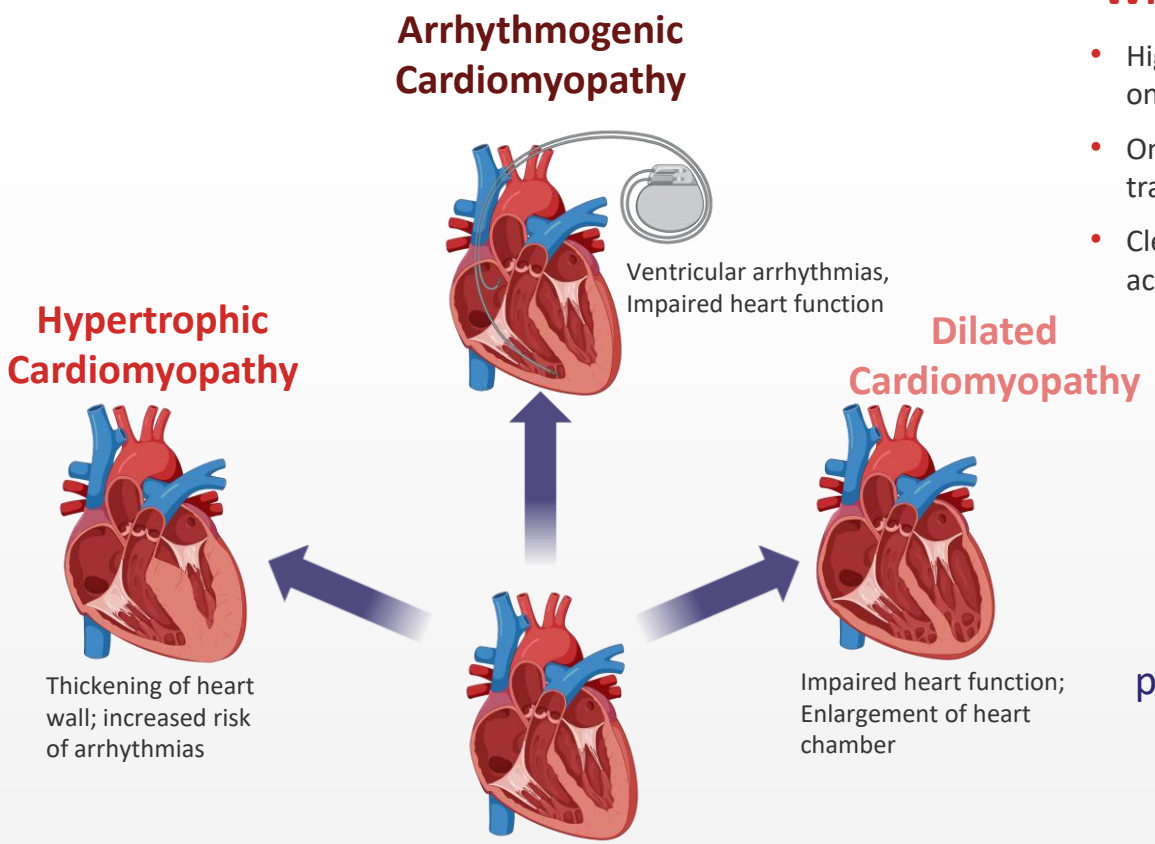
RP-L201: Leukocyte Adhesion Deficiency-I
RP-L102: Fanconi Anemia
RP-L301: Pyruvate Kinase Deficiency

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

AAV, adeno-associated virus; BAG3-DCM, BCL2-associated athanogene 3 dilated cardiomyopathy; HSCs, hematopoietic stem cells; LV, lentiviral vector; DCM, PKP2-ACM, Plakophilin 2 related arrhythmogenic cardiomyopathy

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Well-Positioned as the **Leader in Cardiovascular Genetic Medicines**



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 million**[1]

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

Rocket's Edge:

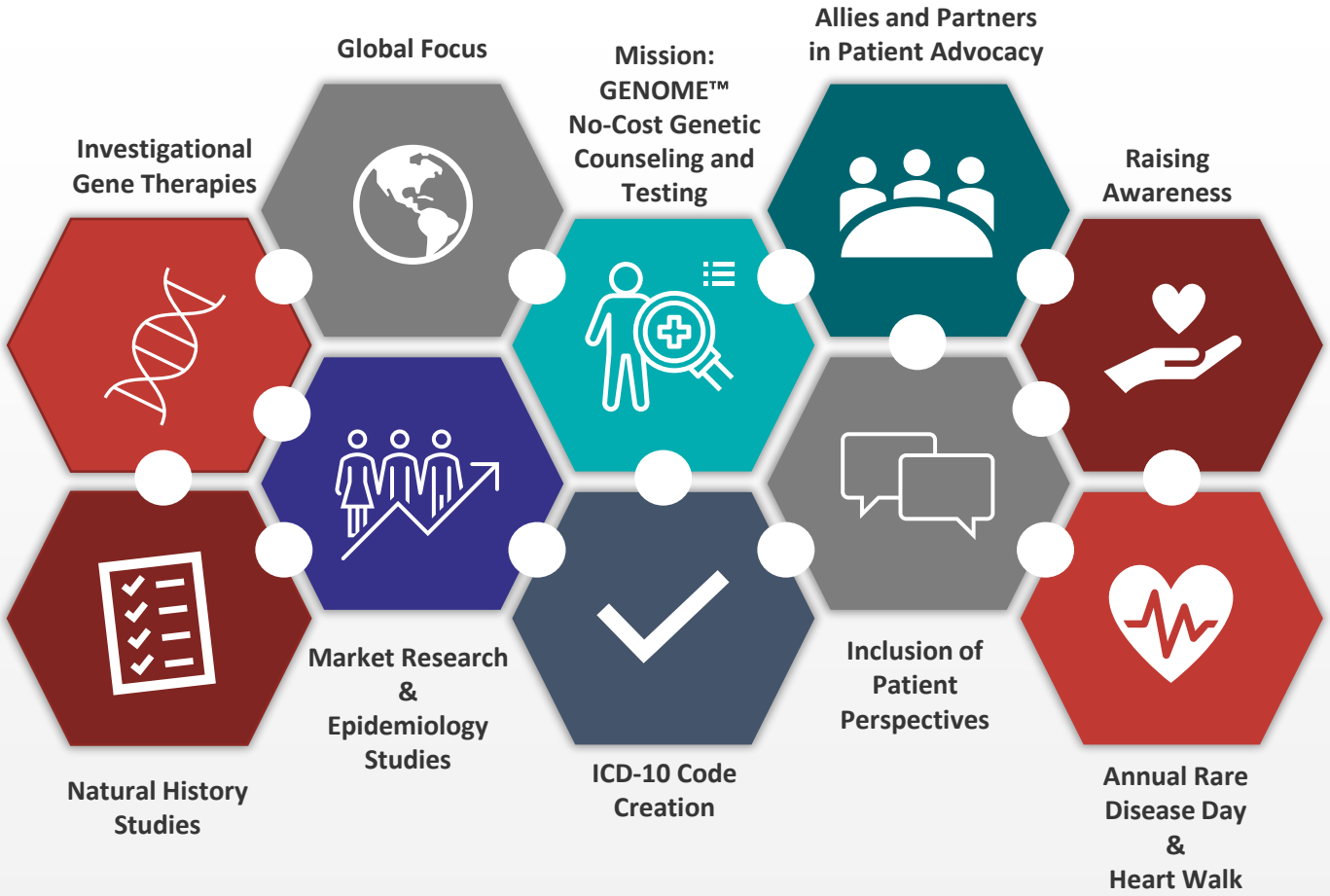
- First company with multi-year efficacy & safety data in cardiac gene therapy
- Proprietary AAV manufacturing
- Experience from treatment of >15 cardiac patients informs late-stage and future AAV programs
- Clinical programs focus on the major phenotypes in inherited cardiomyopathies (**HCM**, **ACM**,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

Inflection Point: We believe advances in genetic understanding and vector delivery now enable **disease-modifying therapies that address the underlying cause**

AAV, adeno-associated virus; ACM, Arrhythmogenic Cardiomyopathy; DCM, Dilated Cardiomyopathy; HCM, Hypertrophic Cardiomyopathy; IV, intravenous

1. Milliman Research Report. Accessed May 2025. https://www.milliman.com/en/insight/2020-us-organ-and-tissue-transplants

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



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

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
- Available treatments do not address the underlying cause of disease
- Only **~20%** of patients receive HTx[1]; Not curative of extracardiac disease

Market Opportunity[2] – US and EU

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

Clinical Manifestations

Impaired autophagy

- Prominent autophagic vacuoles
- Myocardial disarray

Severe cardiomyopathy[1,3]

- Severe left ventricular hypertrophy and life-threatening arrythmias
- 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.

1. Boucek D, Jirikowic J, Taylor M. Natural history of Danon disease. *Genet Med.* 2011;13(6):563-568; 2. Rocket Pharmaceuticals data on file. 3. Brambatti M, Caspi O, Maolo A, et al. Danon disease: Gender differences in presentation and outcomes. *Int J Cardiol*. 2019;286:92-98.

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 evaluated[3]

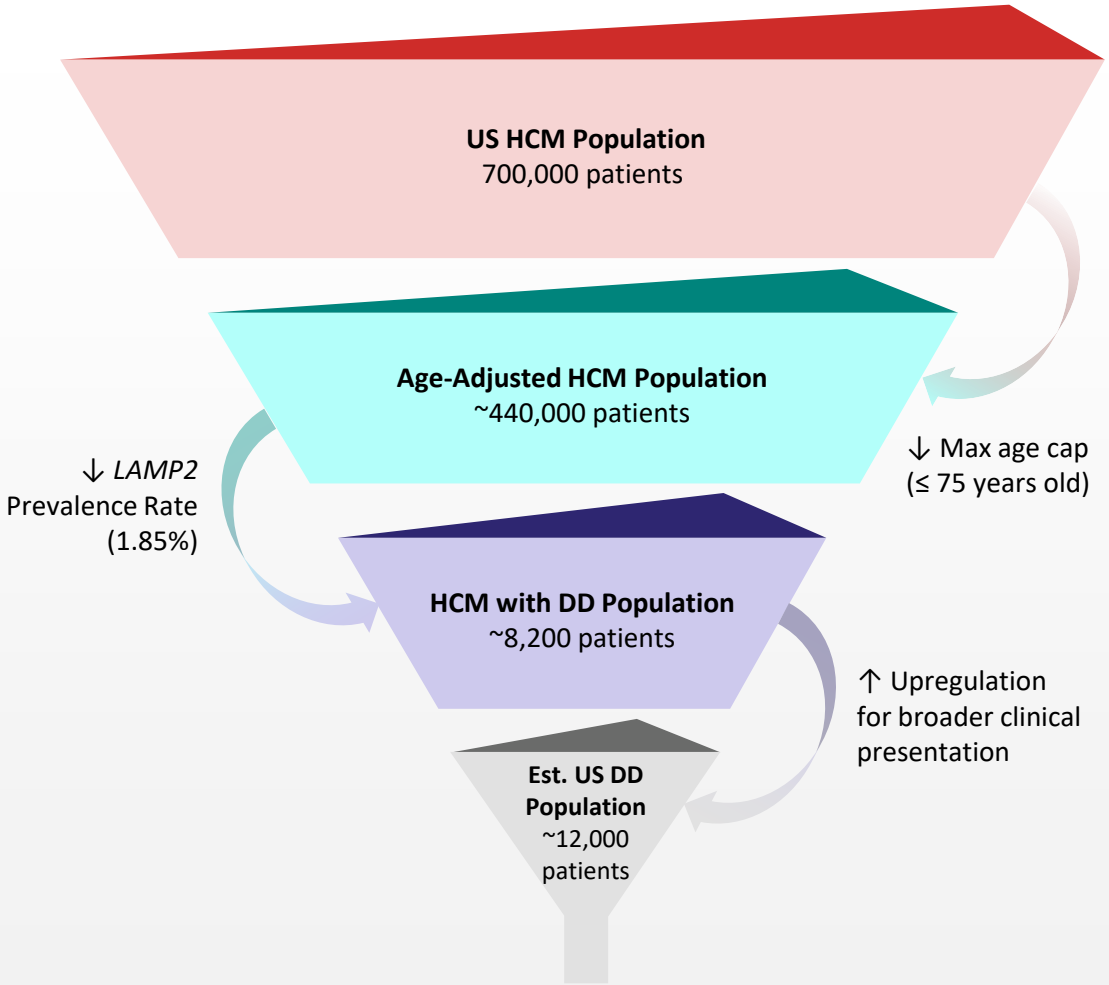
Non-HCM Presenting Danon disease

- 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 code[3]
  - ~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

1. Maron MS, et al. *Circulation.* 2018;138(15):1522-1534.; 2. Massera D, et al. *Int J Cardiol.* 2023;382:64-67; 3. Taylor M, et al. (2025, October 17). *A Hypertrophic Cardiomyopathy-Based Model Estimate of the Prevalence of Danon Disease in the United States.* [Poster abstract]. American Society of Human Genetics 2025 Annual Meeting, Boston, MA, United States.

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Danon Disease can be Recognized Through Multiple Pathways

Cardiac, multisystem, and family-based entry points can converge on a shared path to diagnosis and coordinated care



MULTIPLE ENTRY POINTS

Cardiac findings • Multisystem clues • Family / screening

RECOGNITION & REFERRAL

Connect findings across specialties

GENETIC CONFIRMATION

Testing that includes *LAMP2*

COORDINATED CARE

Specialized monitoring and management

Focused identification opportunity: unexplained HCM in younger males, especially with other Danon-associated features

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

Durable myocardial LAMP2 protein expression seen in all patients

Myocardial LAMP2 Protein Expression

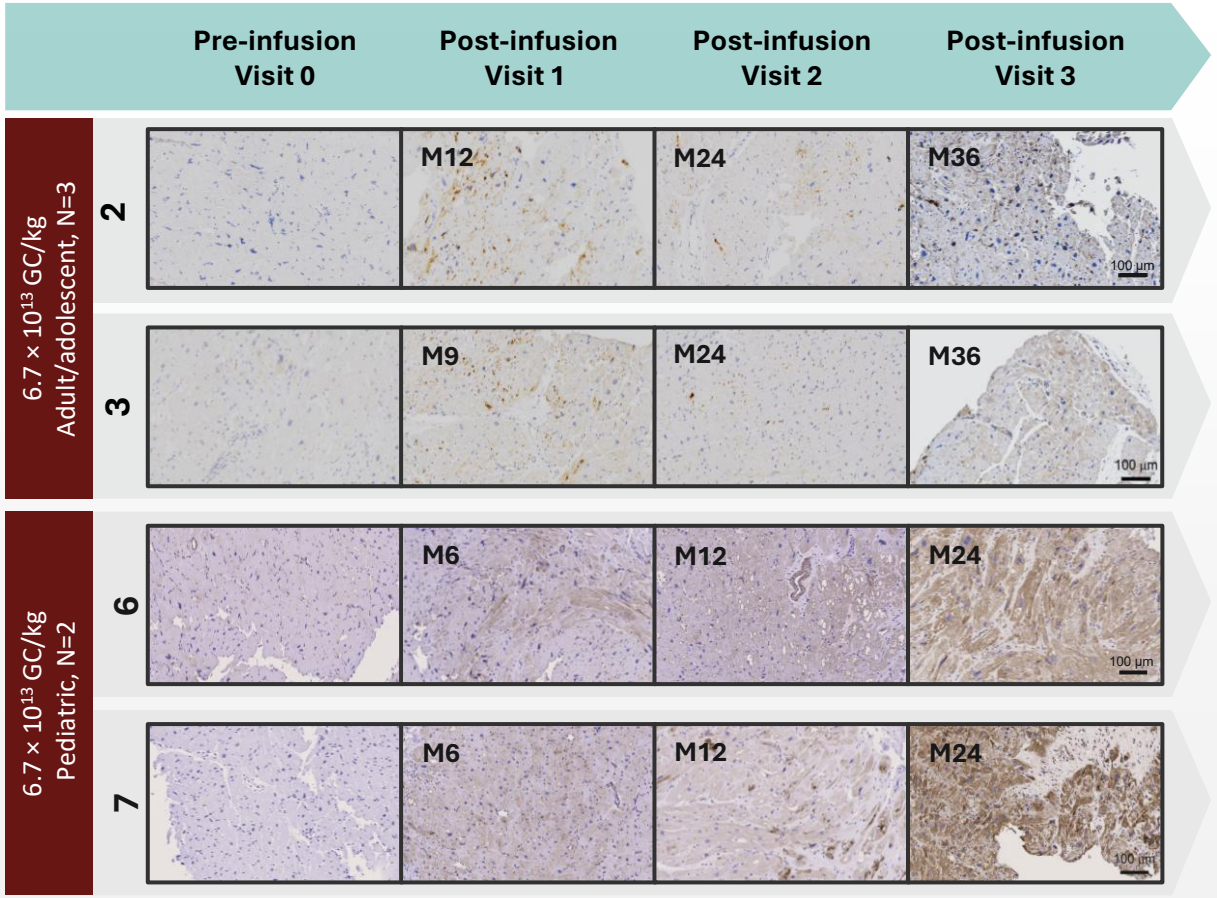
Cohort	Patient	BL	M6	M12	M18	M24	M30	M36	M60
6.7 × 10^{13} GC/kg Adult/adolescent	1	0	++	+	NP	NP	0*	0*	+++‡
	2	0	NP	+++	++	++	++	++	
	3	0	NP	++†	NP	+	+	+	
1.1 × 10^{14} GC/kg Adult/adolescent	4	0	+	+	+	+	NP	+++	
6.7 × 10^{13} GC/kg Pediatric[a]	6	0	++	+	NP	++	Analysis Pending		
	7	0	+	++	NP	+++			

†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 2 (26%–50%)
+++ = Grade 3 (51%–74%)
++++ = Grade 4 (≥75%)
NP = not performed

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
[a]Pediatric patients received 4.1-4.3x10^{13} 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

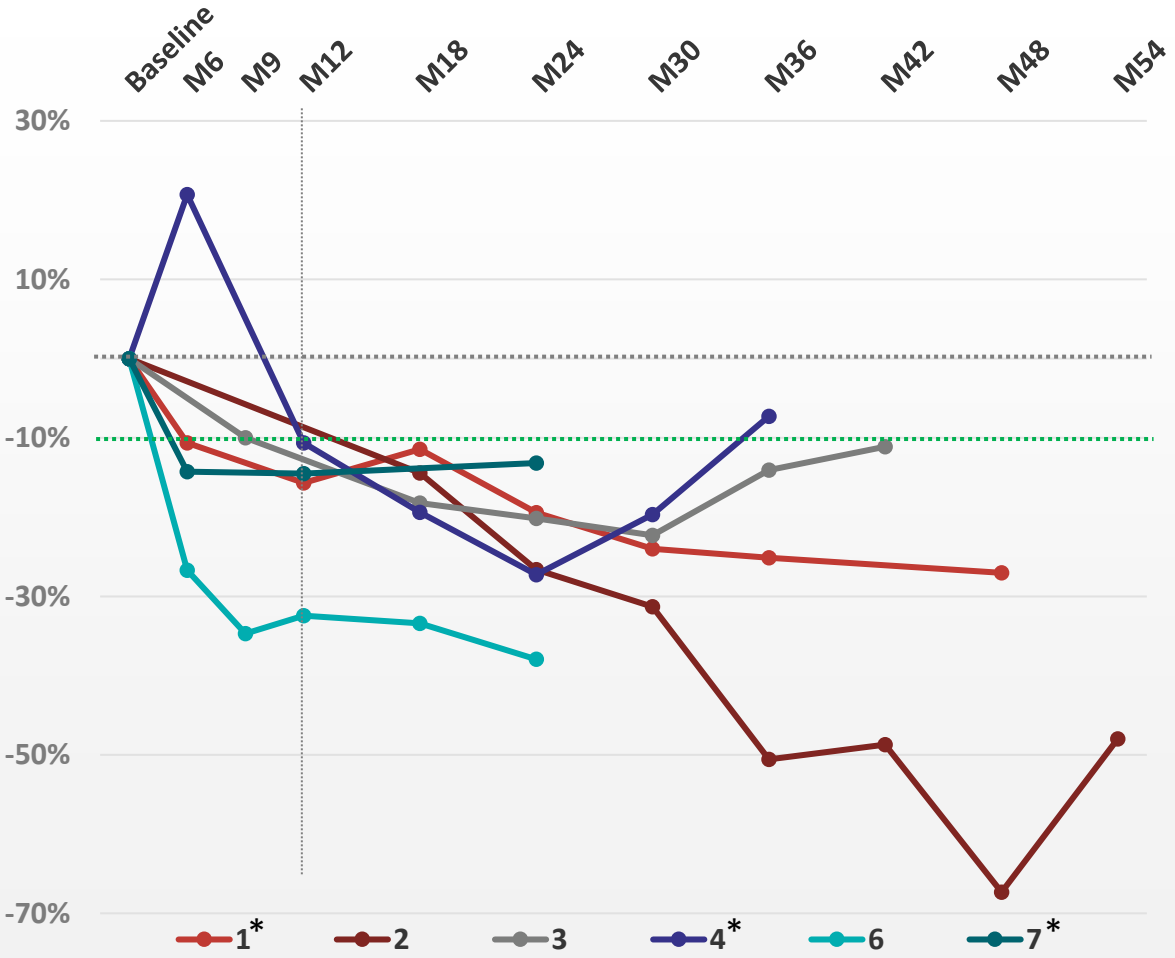
BL, Baseline; BNP, Brain Natriuretic Peptide; cTnI, cardiac troponin I; LAMP2, lysosome-associated membrane protein 2; IVSd, Intraventricular Septum in diastole; KCCQ, Kansas City Cardiomyopathy Questionnaire; LV, Left Ventricle; LVEF, Left Ventricular Ejection Fraction; LVMI, Left Ventricular Mass Index, LVPWd, Left Ventricular Posterior Wall in diastole; m, month(s);MRI, magnetic resonance imaging; NT-Pro-BNP, N-terminal pro–B-type natriuretic peptide; NYHA, New York Heart Association, RV, (Most) Recent Visit; y, year(s)

Data published and used with permission from Greenberg B., et al. *N Engl J Med*. 2025;392(10):972-983; Data cut-off: April 19, 2024. The BNP measure for Patient 9 was at 24 months

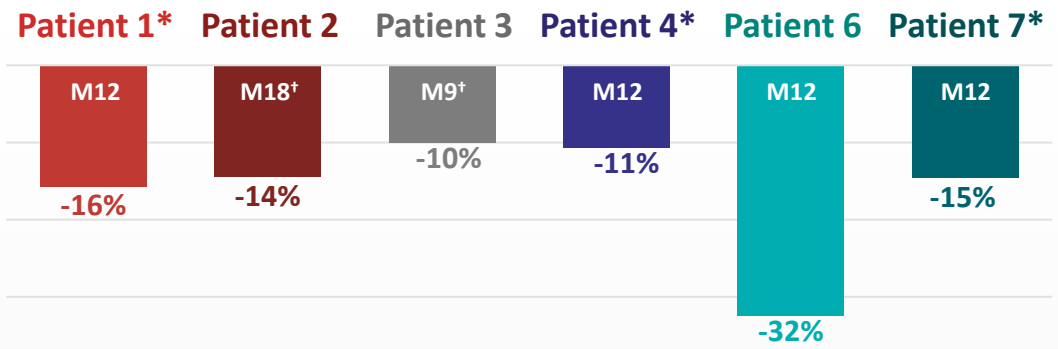
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RP-A501 Phase 1 Study: **Sustained Improvements in LV Mass Index**

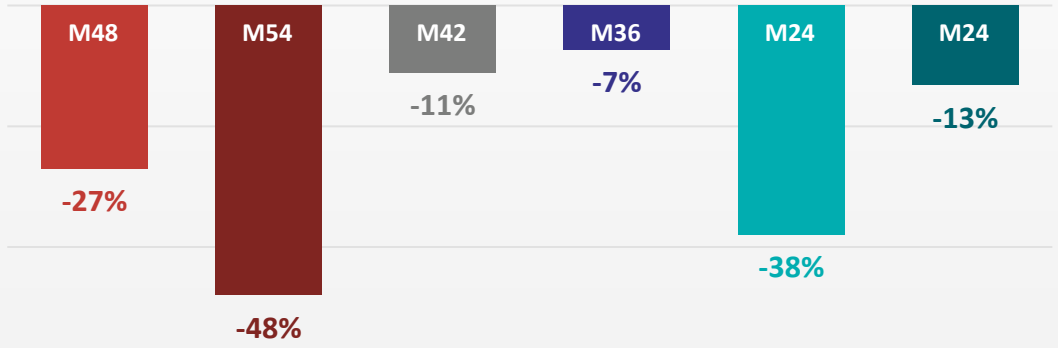
LVMI % Change from Baseline



LVMI % Change: Baseline to 12M†



LVMI % Change: Baseline to Most Recent Visit*



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 18 m data when 12m assessment was not done

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Insights from Danon Disease Patients Treated on the Phase 1 Trial

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

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

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

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

Primary Endpoint Reasonably Likely to Predict Clinical Benefit

Justification for use of LAMP2 protein expression and LV Mass Index

Support for Co-Primary Endpoints

- All components are measurable and unlikely to improve in the absence of a treatment effect
- Primary endpoint will be assessed in the context of QoL, secondary endpoints and concurrent natural history study
- Aligned with FDA expectations for accelerated approval and supported by 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 model[1]
- ***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

LAMP2, lysosome-associated membrane protein 2; LV, left ventricle; MRI, magnetic resonance imaging; QOL, quality of life; WT, wild type

1. Manso et al., *Sci Transl Med.* 2020.

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

Pivotal, global, single-arm, open-label trial[1]

Key eligibility criteria
Males age ≥8 years, *LAMP2* mutation, NYHA II-III, evidence of LV hypertrophy, elevated hsTnI

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
Actively engaging with FDA to align on completing the pivotal Phase 2 trial

Cohort 1

Adult/Adolescent (n=1)
6.7 x 10^{13} GC/kg of RP-A501
IM regimen: Rituximab + Steroid + Sirolimus

Cohort 2

Pediatric (n=3)⁺
6.7 x 10^{13} GC/kg of RP-A501
IM regimen: Rituximab + Steroid + Sirolimus
⁺*Including a 90-day Pediatric Safety Run-in (n=2)*

Cohort 3

Adult/Adolescent and Pediatric with C3 inhibitor (n=2)
6.7 x 10^{13} GC/kg of RP-A501
IM regimen: Rituximab + Steroid + Sirolimus + C3 inhibitor

Cohort 4

Pediatric and Adult/Adolescent
3.8 x 10^{13} GC/kg of RP-A501
IM regimen: Rituximab + Steroid + Sirolimus

Patient 7* Patient 8* Patient 9*

- ✓ **Initial three patients of Cohort 4 were treated safely; RP-A501 was well-tolerated with no TMA or capillary leak syndrome observed**
- ✓ **Safety observations support ongoing FDA engagement regarding the path to treating additional patients and completing the pivotal Phase 2 trial**

*Initial patients received RP-A501 at the recalibrated dose of 3.8 × 10^{13} GC/kg with a refined immunomodulatory regimen and enhanced safety monitoring. Dosing proceeded sequentially, with at least four weeks between infusions. The recalibrated Phase 2 dose was selected to deliver a therapeutic profile consistent with the dose at which RP-A501 demonstrated meaningful efficacy in Phase 1 patients.

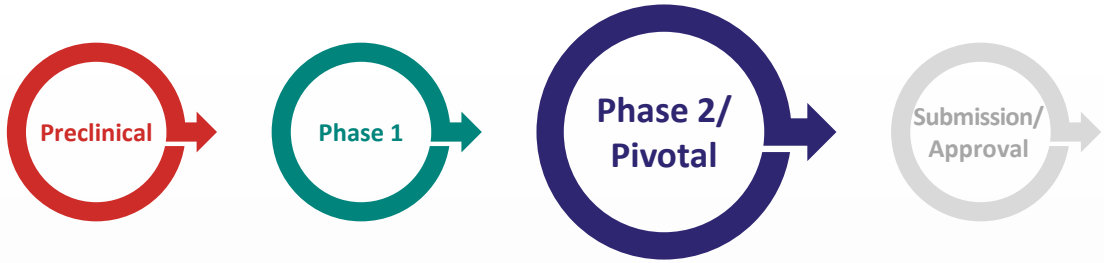
Concurrent Natural History Study[2]

FDA, US Food and Drug Administration; hsTnI, high-sensitivity troponin I; LAMP2, lysosome-associated membrane protein 2; LV, left ventricular; LVMI, left ventricular mass Index; IM, immunomodulatory; NYHA, New York Heart Association; TMA, thrombotic microangiopathy

1. *Gene Therapy Study of RP-A501 in Male Patients With Danon Disease*. ClinicalTrials.gov identifier NCT06092034; 2. *Danon Disease Natural History Study*. ClinicalTrials.gov identifier NCT06214507

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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

- Regulatory update expected in the second half of 2026
- Danon disease Program update expected in the second half of 2026

Regulatory Designations

RMAT and PRIME	Orphan Drug designation in the US	Rare Pediatric Disease designation	Fast Track (US), ATMP

ATMP, Advance Therapy Medicinal Products; PRIME, Priority Medicines; PRV, priority review voucher; RMAT, Regenerative Medicine Advanced Therapy

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 address the underlying cause of disease
- ICD firings are lifesaving but highly traumatic events

Market Opportunity[1] – US and EU Prevalence of **50,000** individuals

Clinical Manifestations

Susceptibility[2]

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

Associated Risks[3]

- 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

ACM, arrhythmogenic cardiomyopathy; ARVC, arrhythmogenic right ventricular cardiomyopathy; ARVD, arrhythmogenic right ventricular dysplasia; ICD, implantable cardioverter defibrillator; LV, left ventricle; PKP2, plakophilin 2; RV, right ventricular; SCD, sudden cardiac death;

1.Rocket Pharmaceuticals data on file; 2. James CA, et al. *J Am Coll Cardiol.* 2013 3. Bhonsale et al. *Eur Heart J.* 2015; 4. Groeneweg et al. *Circ Cardiovasc Genet.* 2015

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

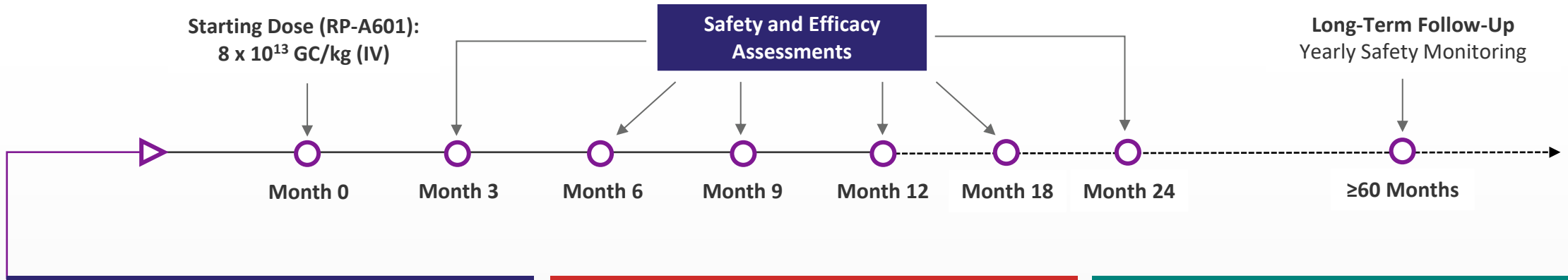
ACM, arrhythmogenic cardiomyopathy; PKP2, plakophilin 2

1. 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
2. McKenna WJ, Judge DP. Epidemiology of the inherited cardiomyopathies. *Nat Rev Cardiol.* 2021;18(1):22-36
3. Rocket Pharmaceuticals data on file

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

First-in-human, multi-center, open-label, dose escalation trial[1]



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 study[2] planned to provide context for the Phase 1 trial and additional information on disease progression

AAVrh, adeno-associated virus rhesus isolate; ACM, arrhythmogenic cardiomyopathy; GC, genome copies; ICD, implantable cardioverter-defibrillator; IV, intravenous; PKP2, plakophilin 2; SAE, serious adverse event (s); TEAE, treatment emergent event(s)

1. *Phase 1, Dose Escalation Trial of RP-A601 in Subjects With PKP2 Variant-Mediated Arrhythmogenic Cardiomyopathy (PKP2-ACM).* ClinicalTrials.gov identifier: NCT05885412; 2. *PKP2-ACM Natural History Study.* ClinicalTrials.gov identifier: NCT06644742

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Preliminary Indications of Improvement or Stabilization with RP-A601

in Clinical Markers of Arrhythmia Burden, Heart Function and QoL

			Heart Function & QoL			Arrhythmia Burden		
Patient	**Age at Enrollment** (y)	**Most Recent Visit** (MRV, m)	**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.

BL, baseline; ECG, electrocardiogram; h, hours; KCCQ, Kansas City Cardiomyopathy Questionnaire; m, month(s); MRV, most recent visit; NSVT, non-sustained ventricular tachycardia; NYHA, New York Heart Association; PVC, premature ventricular contractions; QoL, quality of life; RVEF, right ventricular ejection fraction; y, year(s)

Data cutoff April 2025

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

RMAT	Orphan Drug designations (US and EU)	Fast Track (US)

ECG, electrocardiogram; FDA, US Food and Drug Administration; NSVT, non-sustained ventricular tachycardia; NYHA, New York Heart Association Functional classification; PKP2, Plakophilin-2 Related Arrhythmogenic Cardiomyopathy; PVC, premature ventricular contractions; QoL, quality of life; RMAT, Regenerative Medicine Advanced Therapy; RV, right ventricular; TMA, thrombotic microangiopathy

Cardiovascular Health is Dependent on Functional BAG3 Protein

BAG3-Dilated Cardiomyopathy (BAG3-DCM)



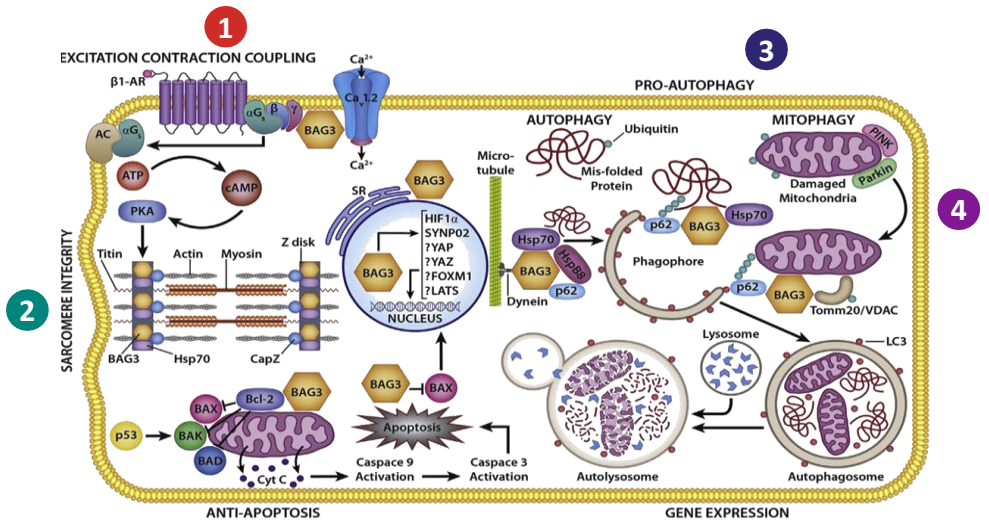
30,000 individuals[1,2] affected by BAG3-DCM in the US

- **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 disease[2]
- Pathogenic variants in BAG3 are estimated to cause **2.3%-6.7%** of DCM cases in the US, Europe, & Japan[2]

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 regulates critical functions in cardiomyocytes[5]



- **Cardiac contractility**
- **Structural support**
- **Protein quality control**
- **Anti-apoptosis**

BAG3, BCL2-associated athanogene 3; BCL2, B-cell lymphoma; DCM, dilated cardiomyopathy;

1. Domínguez et al. *JACC* 2018; 2. Martin et al., *Circulation*, 2024. 3. Hershberger et al., *Nat Rev Cardiol*, 2013; 4. Petretta et al., *Am J Cardiol*, 2011; 5. Myers et al. *JAMA Cardiol*. 2018.

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)



DCM is the most common type of cardiomyopathy

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-up[1]

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

BAG3, BCL2-associated athanogene 3; BCL2, B-cell lymphoma; DCM, dilated cardiomyopathy; ICD, implantable cardioverter defibrillator; LVEF, left ventricular ejection fraction; SOC, Standard of Care

1. Martin et al., *Circulation*, 2024

RP-A701 Restores Cardiac Function Dose-Dependently in a BAG3-KO Mouse Model[1]

Restoration of Cardiac Protein Expression

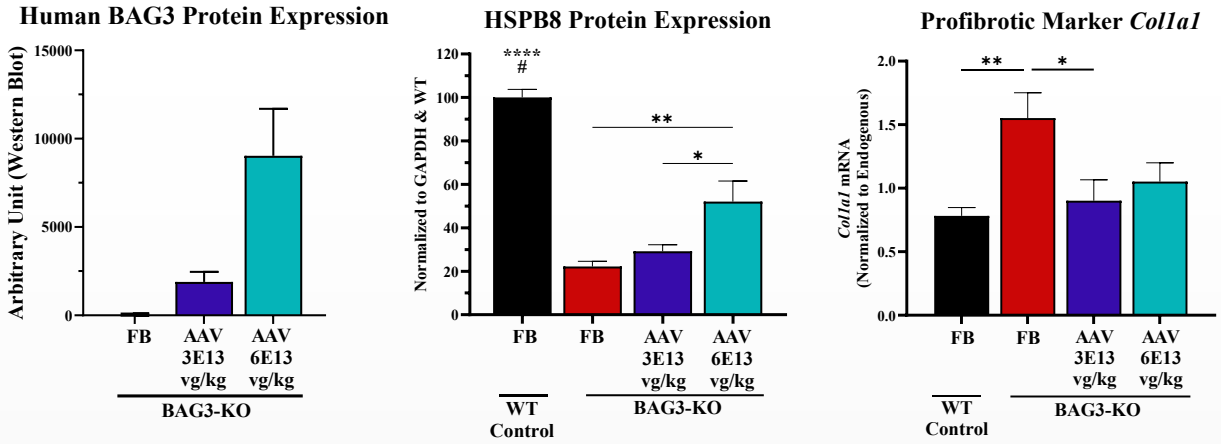
- AAVrh.74-BAG3 produced dose-dependent changes in cardiac protein expression, beyond the BAG3 protein including:
  - Dose-related increase in myocardial HSPB8, a key BAG3 co-chaperone
  - Robust reduction in myocardial expression of the profibrotic marker *Col1a1*

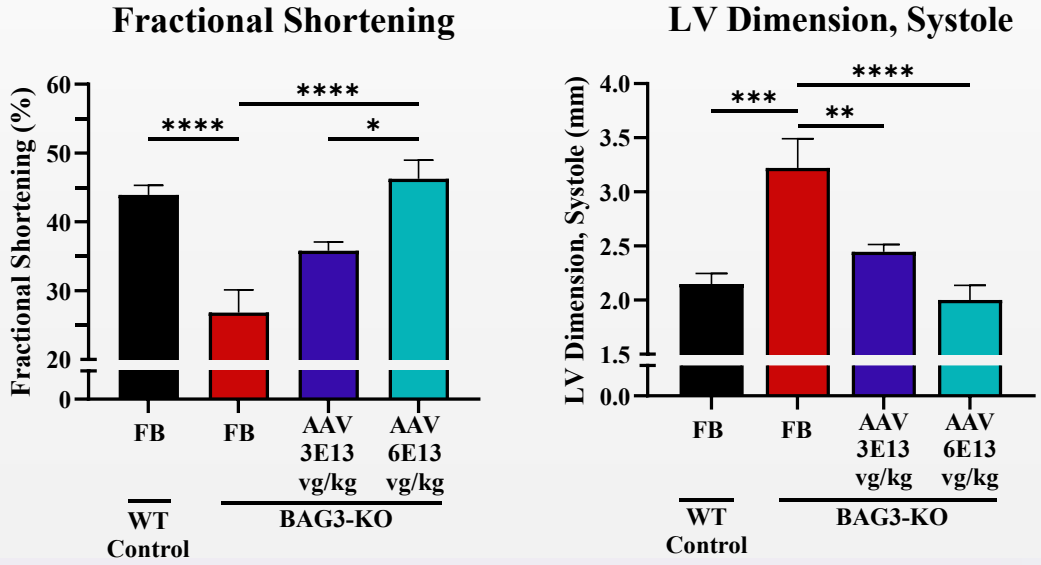
Dose-Dependent Improvement of Cardiac Function

- AAVrh.74-BAG3 Efficacy Study in BAG3 Knockout Mouse Model Post-disease Onset:
  - Dose-related 33–72% improvement in cardiac systolic function (fractional shortening) at 4 months post-infusion
  - Dose-related 23–37% reduction in LV end-systolic dimension at 4 months post-infusion

Favorable Safety Profile

- AAVrh.74-BAG3 Nonclinical Safety Profile:
  - Well tolerated, with no treatment-related adverse effects observed at efficacious doses in WT and BAG3-KO mouse models or at a ≥3-fold higher dose (1.8E14 vg/kg) in NHP studies





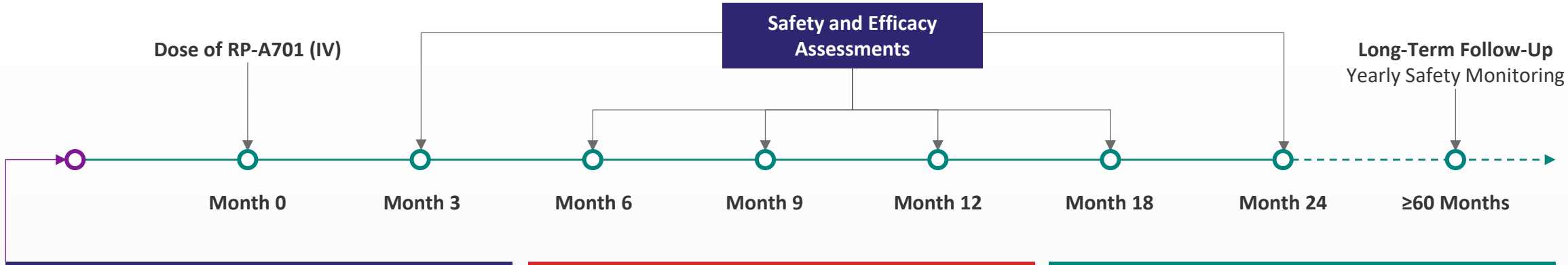
RP-A701 (AAVrh.74-BAG3) is an AAVrh.74-based gene therapy encoding human BAG3 under the control of a cardiac-selective promoter. All nonclinical studies shown used a toxicology lot manufactured using a process representative of the clinical manufacturing process.

[1]Rocket Pharmaceuticals internal nonclinical studies; data on file.

FB, Formulation Buffer; LV, left ventricle; WT, Wild Type

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

First-in-human, open-label, dose escalation trial[1]



INCLUSION CRITERIA

- 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

EXCLUSION CRITERIA

- 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%

ENDPOINTS

Primary Endpoints: Safety

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

Secondary & Exploratory Endpoints: Efficacy

- 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 second half of 2026.

BAG3-DCM, *BAG3* related Dilated Cardiomyopathy; DCM, dilated cardiomyopathy; HF, Heart Failure; ICD, implantable cardioverter-defibrillator; IV, intravenous; NYHA, New York Heart Association; SAE, serious adverse event(s); TEAE, treatment emergent adverse event(s)

1. *Phase 1 AAV Gene Therapy Trial Evaluating Safety and Preliminary Efficacy of RP-A701 in Subjects With BAG3 Dilated Cardiomyopathy.* ClinicalTrials.gov identifier: NCT07137338

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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
- Infections
- Frequent GvHD
- 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 year[4]**)
- 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 Opportunity[1-3] – 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.;

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Now Approved

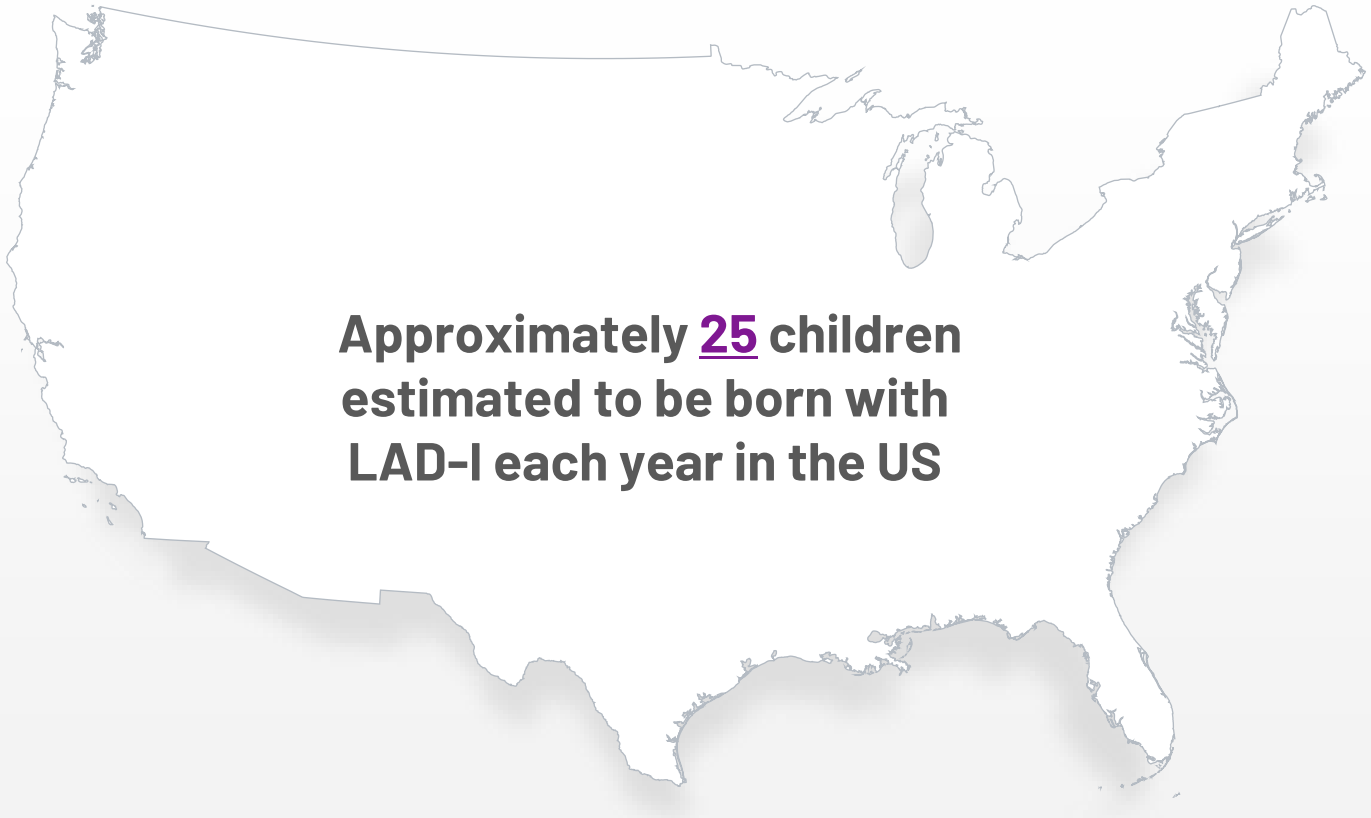
KRESLADI™

marnetegragene autotemcel

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*KRESLADI received FDA accelerated approval based on CD18 and CD11a surface expression. Continued approval may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

Commercial Launch Preparation Activities Ongoing



Commercial Availability Anticipated by Year-End 2026

- Coordinating product supply and readiness with external manufacturing partners
- QTC engagement and activation in progress
- Actively engaging with US payers and policy stakeholders to support coverage
- Given the ultra-rare nature of severe LAD-I, a small number of patients are expected to be treated annually in the U.S. with revenue beginning in 2027

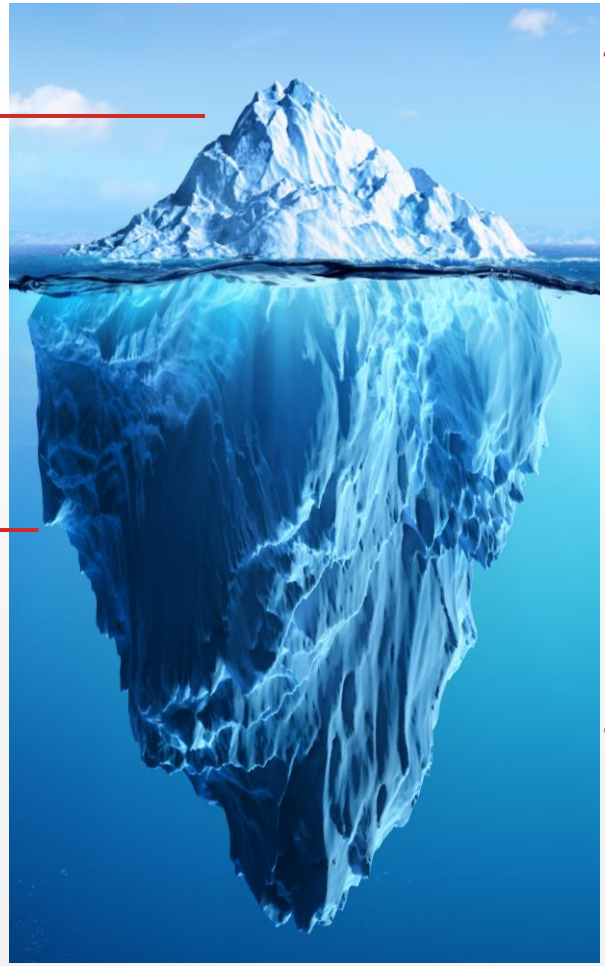
LAD-I, leukocyte adhesion deficiency-I.

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.

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

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Expert Leadership With Proven Track Record



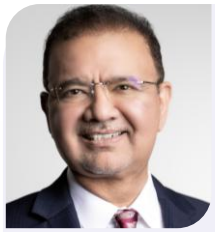
Gaurav Shah, M.D.
Chief Executive Officer
Spearheaded Kymriah development at Novartis towards approval.

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



Sarbani Chaudhuri
Chief Commercial & Medical Affairs Officer
20+ years of experience driving commercial growth for rare cardiac and hematology launches.

Johnson&Johnson · AstraZeneca · Pfizer · NOVARTIS



Syed Rizvi, M.D.
Chief Medical Officer
20+ years of experience across all stages of drug development.

POSEIDA THERAPEUTICS · CARIBOU BIOSCIENCES · CHIMERIC THERAPEUTICS · LEGEND BIOTECH



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

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



Chris Stevens
Chief Operating Officer
25 years of proven success in technical operations, product strategy, and leadership.

Spark Therapeutics · GSK · Bristol Myers Squibb



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

endo · PAR · Teligent · ...ichnos...



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.

Krystal · equilibre · GRAVITON

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THANK YOU!



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