

Rocket Pharmaceuticals

Corporate Overview

November 2026

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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 actual results to differ materially from those expressed or implied by such forward-looking statements. Rocket makes these statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. Forward-looking statements 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; its ability to obtain additional funding to conduct planned research and development efforts; the potential safety and effectiveness of RP-A501, including at the recalibrated dose under the modified protocol; its ability to continue enrollment and dosing, complete the pivotal study and achieve its prespecified efficacy success criterion; the expected timing and results of ongoing and planned clinical trials; the expected timing and outcome of regulatory interactions and submissions, including a potential biologics license application seeking accelerated approval; Danon disease prevalence and addressable patient estimates; patient identification and referral efforts; potential commercial opportunity and revenue; commercialization plans, including the development of sales and marketing capabilities and relationships with treatment centers and other third parties; and potential development in additional patient populations.

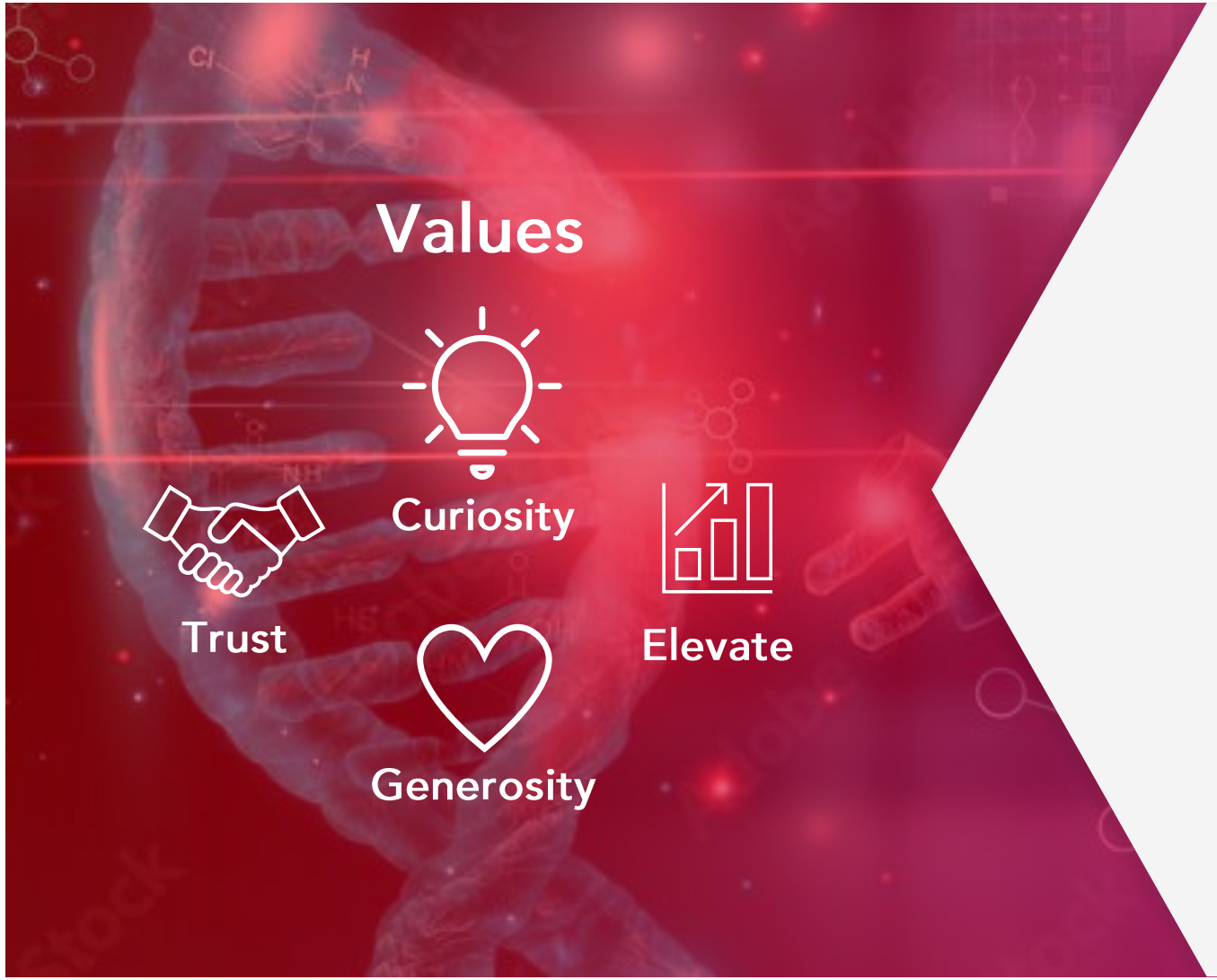
Although Rocket believes that the expectations reflected in these forward-looking statements are reasonable, it cannot guarantee such outcomes. Actual results may differ materially as a result of various important factors, including, without limitation, clinical trial results and safety findings, including the risk of additional serious adverse events; Rocket's ability to identify and enroll eligible patients and successfully complete clinical studies; the possibility that earlier clinical observations or nonclinical findings may not predict subsequent clinical outcomes; changes in regulatory requirements or expectations; Rocket's ability to obtain regulatory approval and meet applicable post-approval requirements; manufacturing, product-quality and supply risks; dependence on third parties for development, manufacture, marketing, sales and distribution; the availability of sufficient funding and unexpected expenditures; the accuracy of population estimates and assumptions underlying commercial models; pricing, reimbursement, market acceptance and competition; the outcome of litigation; Rocket's ability to achieve the expected benefits of its portfolio prioritization and strategic restructuring; and its ability to obtain and enforce patents and defend against third-party infringement claims. Additional risks are described 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 Securities and Exchange Commission, and subsequent filings with the SEC, including its Quarterly Reports on Form 10-Q.

RP-A501 is investigational, and its safety and efficacy have not been established. Clinical observations presented here are based on small, uncontrolled studies with varying follow-up. Preliminary findings may change as additional data become available, and results from earlier cohorts may not predict outcomes at the recalibrated dose under the modified protocol. Meeting the pivotal study's efficacy success criterion would not, by itself, establish that RP-A501 will receive regulatory approval. FDA's assessment would consider the complete application, including safety, efficacy, manufacturing and product-quality information. Danon disease prevalence and addressable patient estimates are based on available data and assumptions and are subject to uncertainty. Estimated prevalence and identified patient records do not establish the number of patients eligible for treatment. Revenue illustrations are internal modeling scenarios, rather than financial guidance, and depend on clinical success, regulatory approval, the approved patient population, patient identification, pricing, reimbursement and treatment uptake. Opportunities in additional patient populations would require further development and applicable regulatory approvals.

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, except as required by law.

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Vision: Seeking Genetic Medicines Cures



Mission

To develop first-in-class and best-in-class curative genetic medicines for patients with life-threatening diseases

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A Fully Integrated Cardiovascular Rare Disease Company

Integrated science, manufacturing and commercial capabilities to transform the lives of patients with life-threatening cardiovascular diseases

LATE-STAGE SCIENCE & INNOVATION

First-and-best-in-class approach to treating complex and life-threatening genetic disorders

First Company with genetic medicine safety and efficacy data **targeting the heart**

INTEGRATED MANUFACTURING

US-based in-house cGMP manufacturing built for quality, scale and speed

~100,000 sq ft US-based in-house AAV cGMP facility

EXPERIENCED LEADERSHIP

Proven track record of delivering therapies from development to patients

20+ US and ex-US approvals and launches

FINANCIAL STRENGTH

Well capitalized to advance our full pipeline and deliver long-term value

$279.4M[1] cash and cash equivalents; expected runway into 3Q 2028

FOUNDATIONAL STRENGTHS

- Late-stage science and innovation
- World-class scientific and commercial expertise
- Strategic partnerships with patient communities, HCPs and payors
- End-to-end capabilities, from discovery to patient access

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

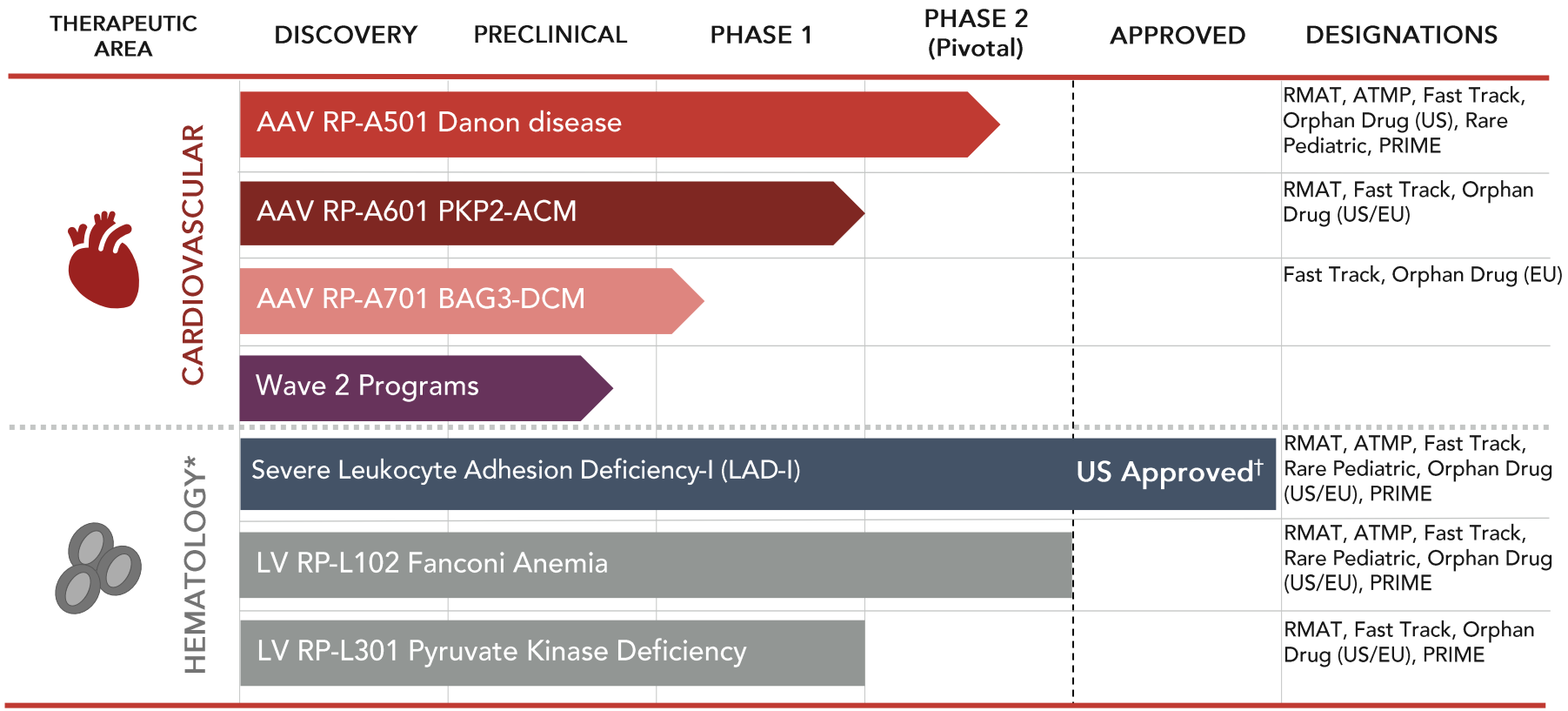
[1]Cash balance as of September 30, 2026. Borrowings under the Hercules facility could extend runway into 2029, subject to applicable borrowing conditions.

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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

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Rocket Offers Multi-platform Gene Therapy Expertise

IN VIVO platform

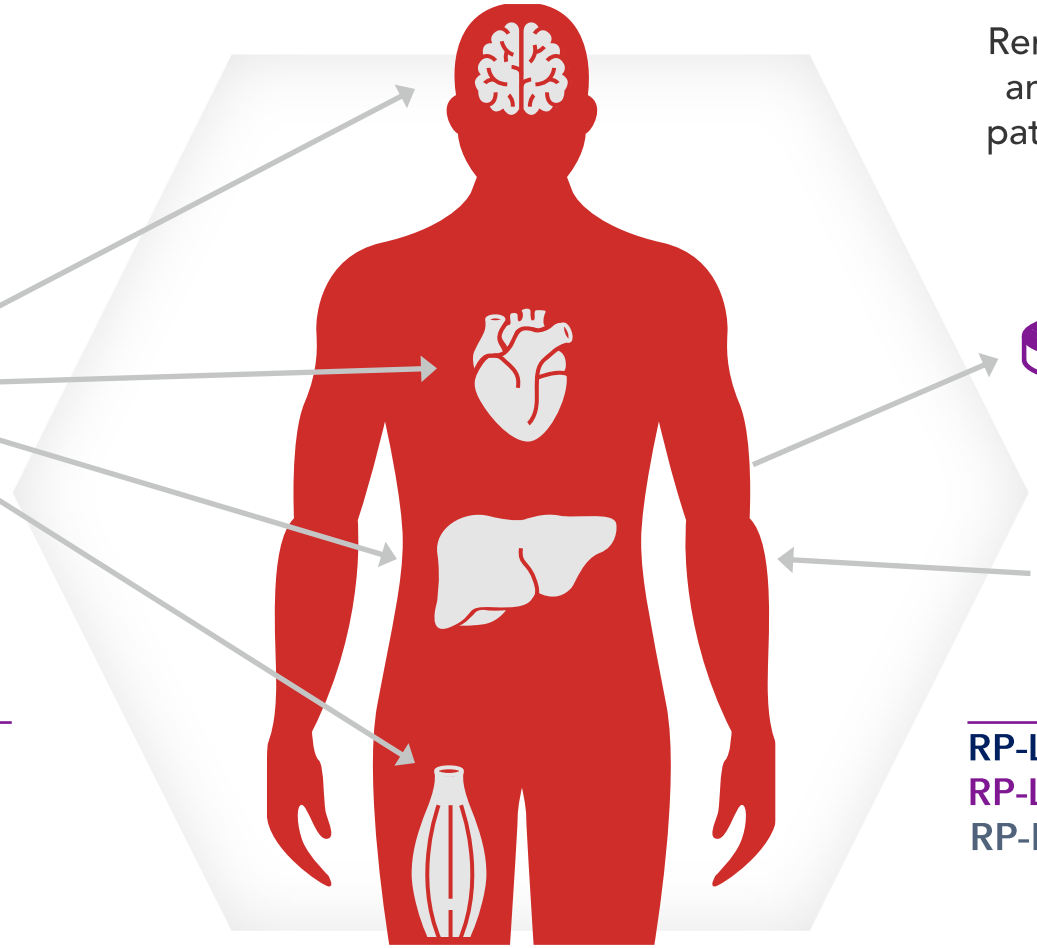
Laboratory-produced AAV

Therapeutic AAV



Direct intravenous injection

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

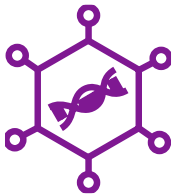


EX VIVO platform

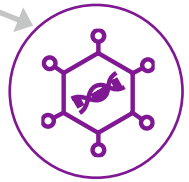
Remove cells and isolate patient HSCs



Laboratory-produced LV



Therapeutic LV



Gene-modified HSCs

Infusion of modified HSCs

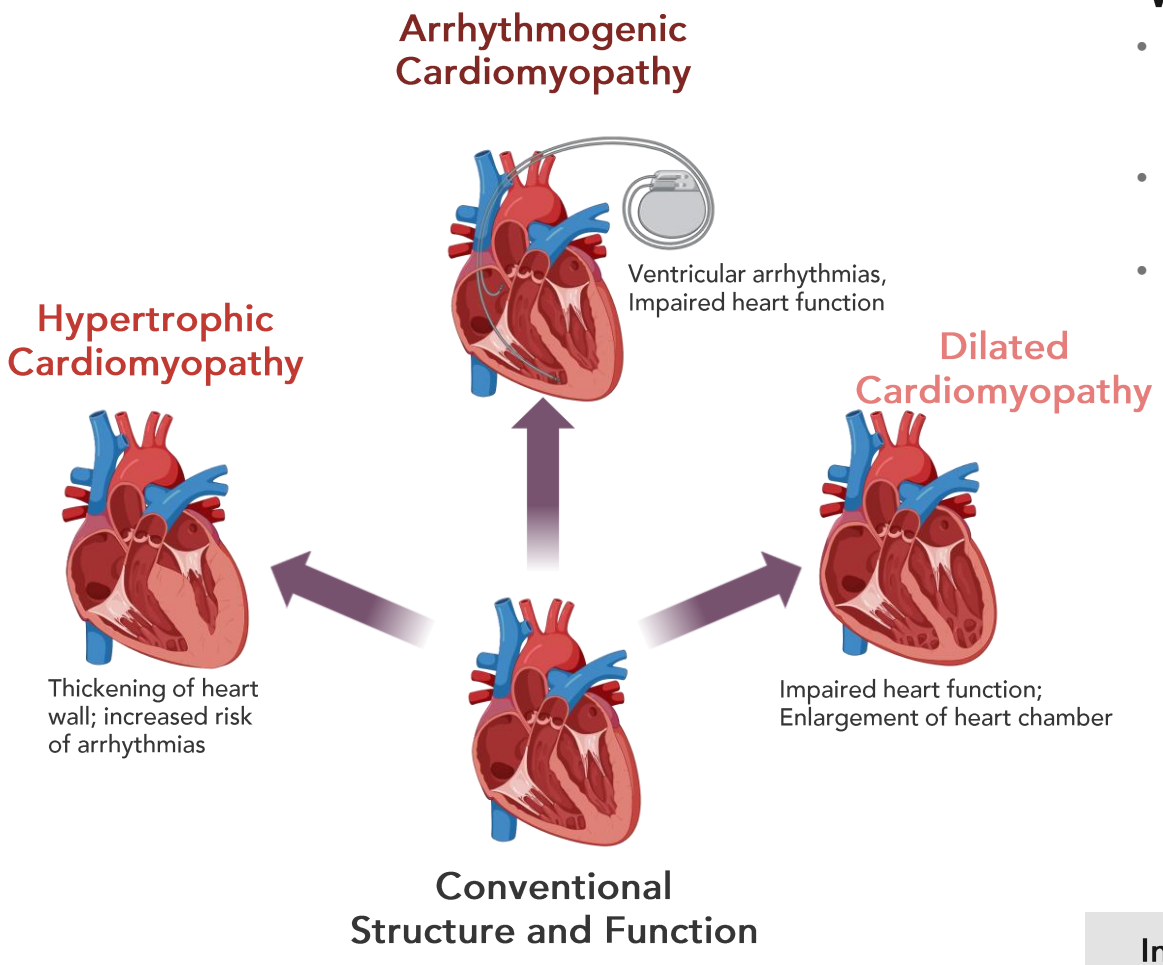
RP-L201: Leukocyte Adhesion Deficiency-I
RP-L102: Fanconi Anemia
RP-L301: Pyruvate Kinase Deficiency

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
- Growing clinical experience across multiple cardiac gene therapy programs informs late-stage and future AAV development
- 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

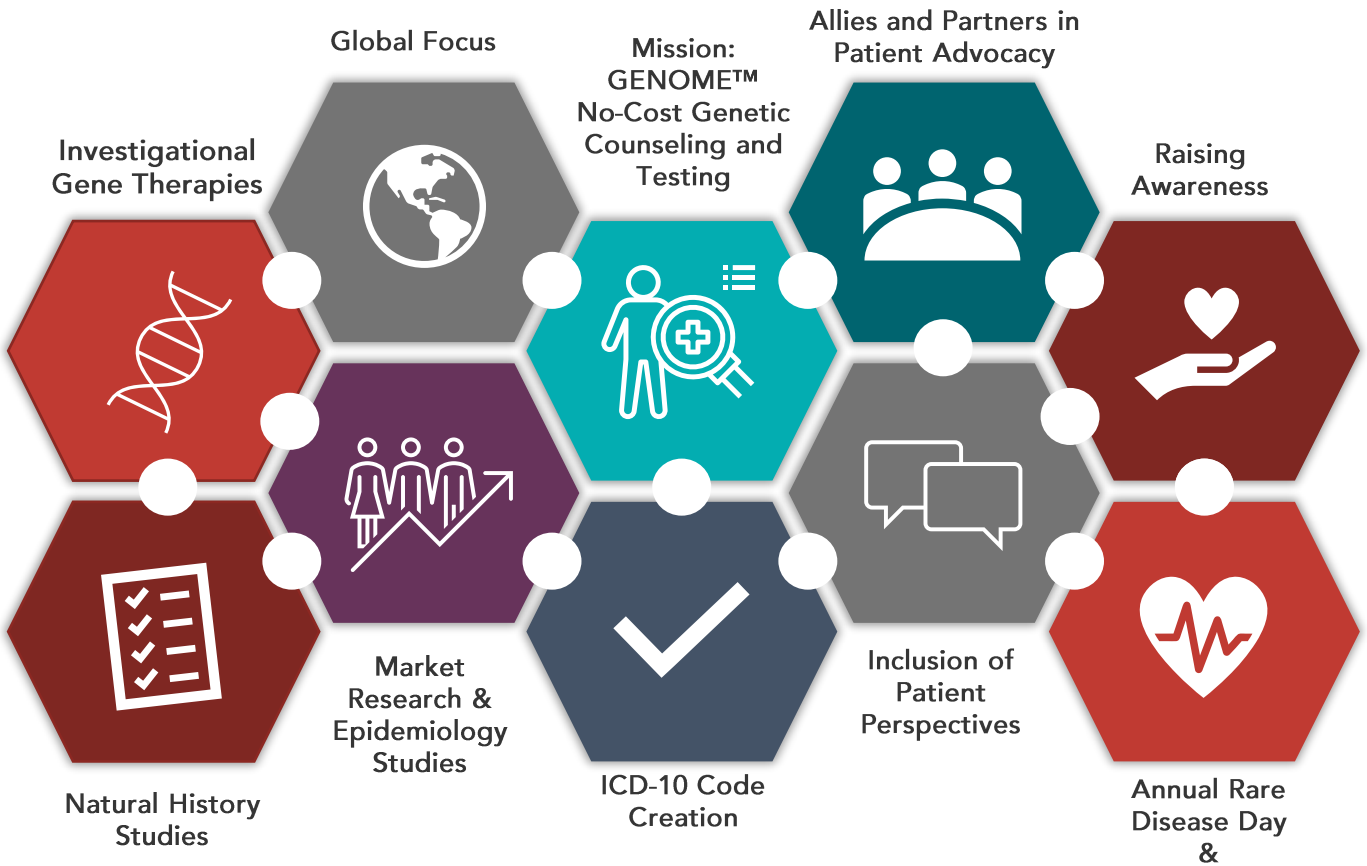
Inflection Point: We believe advances in genetic understanding and vector delivery now enable **disease-modifying therapies that address the underlying cause**

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

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Our Commitment to the Rare Disease Community





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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.

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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 ~25% of patients receive HTx[1]; Not curative of extracardiac disease

Market Opportunity[2] – US and EU

Prevalence of 20,000 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.

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Estimated Epidemiology of Danon Disease is 20K+ across US & EU5

Independent genotype and phenotype-based analyses converge on similar estimate of 10-11K US patients

Genotype-Based Analysis

Pathogenic & likely Pathogenic LAMP2 Variants

Based on comprehensive molecular characterization of confirmed Danon Disease + population genomic anchor

62% **(~6.6K)**

Anchor

Classic loss-of-function *LAMP2* variants

Population allele frequency of 3 variant categories[1] from gnomAD[2]

38% **(~4.1K)**

Scaling

Other P/LP *LAMP2* variants

6 variant categories[3] identified through RW datasets[4] with confirmed DD

450+ patient records

9+ variant categories

ESTIMATED DANON POPULATION

10-11K PATIENTS

~4K Male | ~6-7K Female

Phenotype-Based Analysis

Natural History and Real-World Modeling

Based on Danon disease natural history modeling + real-world patient data

~5.1K

Anchor

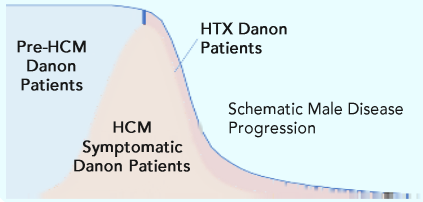
Patients with HCM Phenotype[5]

Age & sex-specific DD yield modeling with HCM population

~5.4K

Scaling

Pre-cardiomyopathy and DCM Patients



Age- and sex-specific natural history model built with 700+ patient data

DCM, dilated cardiomyopathy; DD, Danon disease; EU5; Germany, France, Italy, Spain, UK; HCM, hypertrophic cardiomyopathy; HTx, heart transplantation; P/LP, pathogenic/likely pathogenic; RW, real-world;

1. Stop-gain (Nonsense), Frameshift and Canonical splice-site variant categories 2. non-UKBB gnomAD v4.11 1. non-UKBB gnomAD v4.11 Genome Aggregation Database 3. includes Start-loss, Exon/gene deletions/duplications, Missense, Synonymous and Intronic variant categories 4. variant records of confirmed DD patients from genetic testing labs Invitae, Ambry Genetics and GeneDx, real-world Natural History Cohorts, Rocket Natural History Study, Repository of published RW Danon patient cases 5. HCM & DD claims/EHR databases (Cellworks, Forian) covering >310M lives

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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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Phase 1 and Phase 2 Study of RP-A501 in Danon Disease

Global, single-arm, Pivotal Phase 2 Trial of RP-A501 ongoing; 12-Patients aligned with FDA

Phase 1: 3 years (completed)

Cohort 1
Adult/Adolescent (n=3)
6.7 x 10^{13} GC/kg RP-A501
IM regimen: Steroid (n=1); Steroid + Tacrolimus (n=2)

Cohort 2
Adult/Adolescent (n=2)
1.1 x 10^{14} GC/kg RP-A501
IM regimen: Rituximab + Steroid + Tacrolimus

Cohort 1A
Pediatric (n=2)
6.7 x 10^{13} GC/kg RP-A501
IM regimen: Rituximab + Steroid + Sirolimus

Phase 1 Results in NEJM '25[1]

Phase 1 LTFU: up to 10 years (ongoing)

Pivotal Phase 2 (ongoing)[2]

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

Cohort 2
Pediatric (n=3)+
6.7 x 10^{13} GC/kg RP-A501
IM regimen: Rituximab + Steroid + Sirolimus
+Included initial sequential Pediatric Safety Run-in (n=2)

Cohort 3
Adult/Adolescent and Pediatric with C3 inhibitor (n=2)
6.7 x 10^{13} GC/kg RP-A501
IM regimen: Rituximab + Steroid + Sirolimus + C3 inhibitor

Cohort 4
Adult/Adolescent and Pediatric (n=12)
3.8 x 10^{13} GC/kg RP-A501
IM regimen: Rituximab + Steroid + Sirolimus

← *Pivotal Phase 2 Trial*

Current Status:
Enrolling and treating remaining Cohort 4 patients (n=9) in Pivotal Phase 2 Study

Co-Primary Endpoints (at 12 months):
- Improvements in LAMP2 protein expression (≥ Grade 1 from baseline)
- Reductions in Left Ventricular Mass (LVMI; ≥10%)

IM, immunomodulatory regimen; LAMP2, lysosome-associated membrane protein 2; LTFU, Long-term follow-up; LV, left ventricular; LVMI, left ventricular mass Index

1. Greenberg B., et al. *N Engl J Med.* 2025;392(10):972-983; 2. *Gene Therapy Study of RP-A501 in Male Patients With Danon Disease.* ClinicalTrials.gov identifier NCT06092034

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RP-A501 Phase 1 Study: Sustained Cardiomyocyte LAMP2 Expression

Durable myocardial LAMP2 protein expression seen in all patients

Myocardial LAMP2 Protein Expression

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

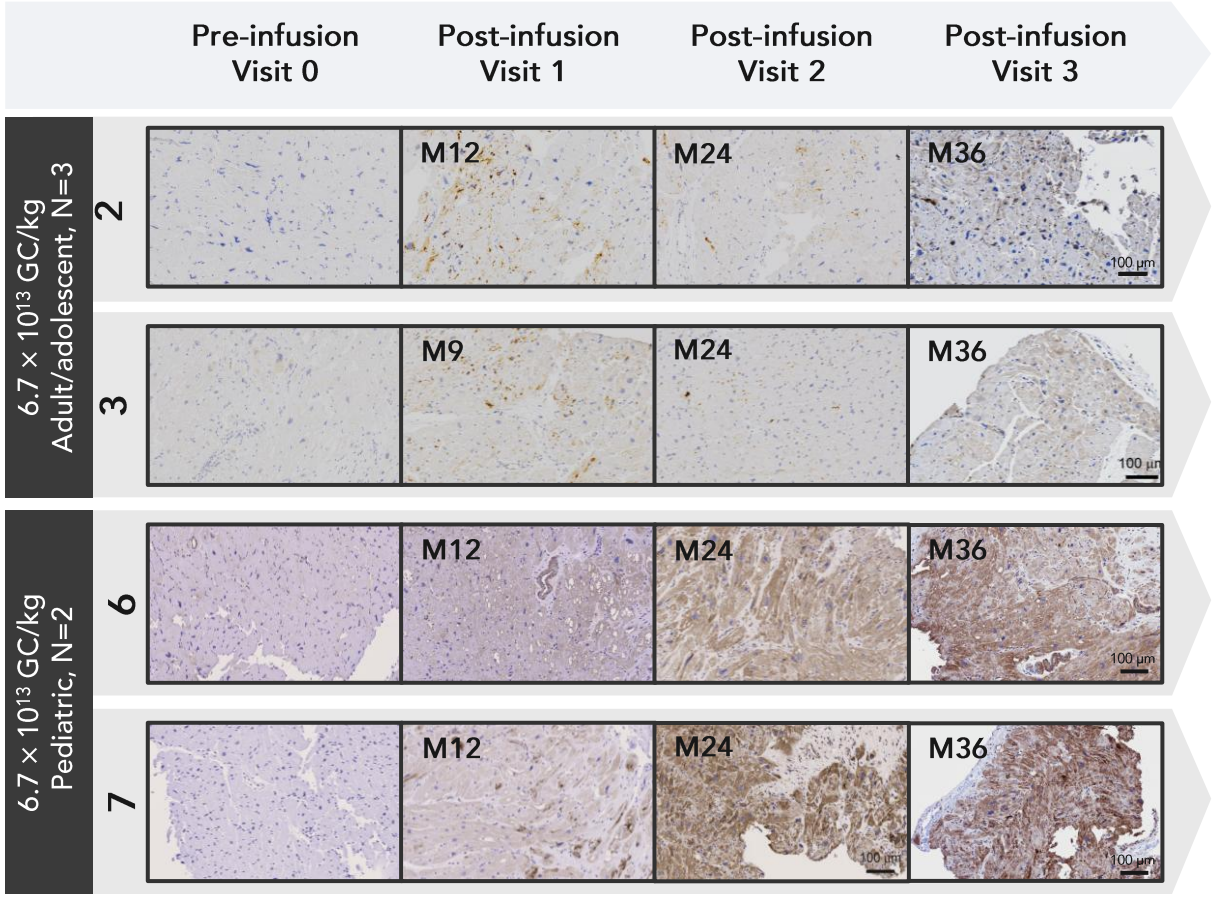
Legend: IHC Staining Grade¶ (% Positive Cardiomyocytes)

Grade 0 = no staining; + = Grade 1 (≤25%); ++ = Grade 2 (26%–50%); +++ = Grade 3 (51%–74%); ++++ = Grade 4 (≥75%)

NP = not performed

†Reflects 9M visit biopsy as 12M biopsy not performed.
*Grade 0 LAMP2 protein IHC staining at the 30- and 36-month assessments, however, LAMP2B vector RNA and DNA (VCN) levels have persisted through 60 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.
*Patient 5 had LV systolic dysfunction (LVEF <40%) at enrollment and had progressive heart failure requiring transplantation 5m following RP-A501 treatment and is not evaluable for efficacy.

Representative LAMP2 IHC Images



BL, baseline; IHC, Immunohistochemistry; LAMP2, lysosome-associated membrane protein 2; m, month(s);
Data Extraction Date: July 31, 2026

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RP-A501 Phase 1: Benefit Observed Across All Key Parameters up to 7 years

Lower cardiac biomarkers accompany stable or reduced LV mass index and improved quality-of-life scores

Cohort	Patient	Age at Most RV (y)	Most Recent Visit (m)	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, BL to RV
1:Low Dose Adult/ Adolescent	1	24	84‡	-32%, 85.0 to 57.8	+2%, 19.8 to 20.2	-30%, 18.8 to 13.2	-31%, 336 to 233	-99%, 0.60 to 0.01	II to I	+56, 44 to 100
	2	26	72	-31%, 260.2 to 178.9	-29%, 60.1 to 42.6	-71%, 39.1 to 11.3	-90%, 5119 to 489	-96%, 1.46 to 0.06	II to I	+24, 64 to 88
	3	23	54‡	-12%, 98.2 to 86.7	-30%, 30.9 to 21.7	-56%, 32.1 to 14.1	-45%, 841 to 460	-76%, 0.28 to 0.07	II to II	+7, 77 to 84
2:High Dose Adult/ Adolescent	4	23	36	-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[a]	15	36	-47%, 141.5 to 74.7	-32%, 42.4 to 29.0	-2%, 22.8 to 22.3	-75%, 1629 to 406‡	-84%, 1.78 to 0.28	II to I	+13, 50 to 63
	7	14	36	+3%, 82.0 to 84.9*	+13%, 18.5 to 20.9	+66%, 14.9 to 24.7	-35%, 1912 to 1238	-31%, 1.08 to 0.74	II to I	+36, 52 to 88

*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 was performed on cryopreserved and non-cryopreserved samples. Values for cTnI from high-sensitivity and earlier tests. High-sensitivity and earlier assays are expressed in ng/mL.
‡ For Patient 1, NYHA class and KCCQ-12 assessments occurred at Month 84, and all other assessments at Month 72. For Patient 3, NYHA class assessment occurred within the Month 84 visit window, and all other assessments at Month 54.
[a]Patient underwent heart transplant at 4.8 years post-RP-A501 infusion; post data cut.

Improved | Stabilized | Worsened

BL, Baseline; BNP, Brain Natriuretic Peptide; cTnI, cardiac troponin I; LAMP2, lysosome-associated membrane protein 2; IVSd, Interventricular 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 Extraction Date: July 31, 2026

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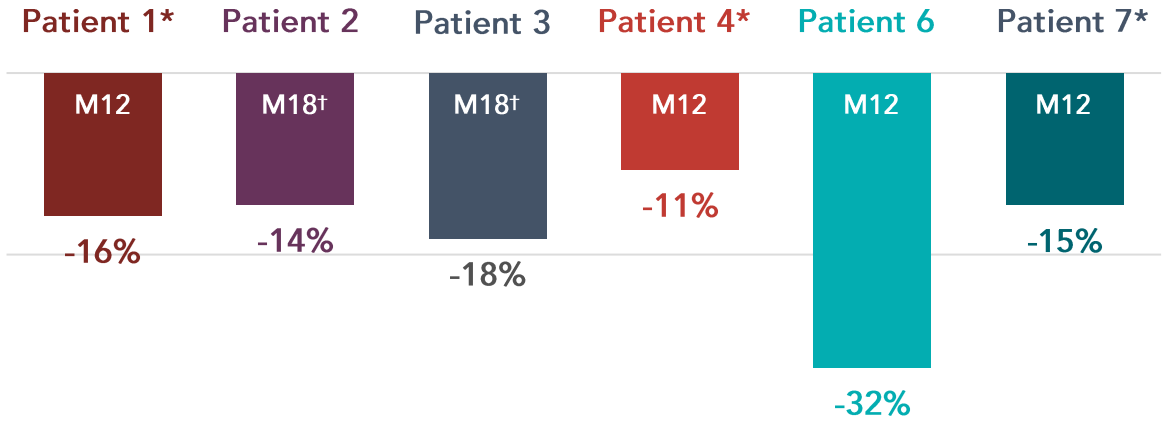
RP-A501 Phase 1 Summary

LAMP2 expression and LVMI improvements seen as early as 6 months post RP-A501

Myocardial LAMP2 Protein Expression

Cohort	Patient	BL	M6	M12
6.7 × 10^{13} GC/kg Adult/adolescent	1	0	++	+
	2	0	NP	+++
	3	0	NP	++ ‡
1.1 × 10^{14} GC/kg Adult/adolescent	4	0	+	+
6.7 × 10^{13} GC/kg Pediatric	6	0	++	+
	7	0	+	++

LVMI % Change: Baseline to 12m†



Summary of Phase 1 in Relation to Pivotal Co-Primary Endpoints

- All patients showed ≥10% LVMI decrease and LAMP2 protein expression increase (≥ Grade 1) at ~12m, representing 100% response rate based on pivotal Ph 2 endpoints
- All patients showed durable myocardial LAMP2 protein expression and improved or sustained LVMI at most recent visit

* Where possible, cardiac MRI assessments shown (patients 1, 4, and 7); otherwise, echocardiogram data presented.
† Utilized 18 m data when 12m assessment was not done. ‡Reflects 9M visit biopsy as 12M biopsy not performed.
LAMP2, lysosome-associated membrane protein 2; LVMI, left ventricular mass index; MRI, magnetic resonance imaging; m, month(s).
Data Extraction Date: July 31, 2026

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RP-A501 Pivotal Phase 2 Trial Design

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

- ✓ Cohort 1-3 completed
- ✓ FDA agreement obtained to support completion of pivotal study
- ✓ Pivotal data package: 12 patients at recalibrated dose + revised immunomodulatory regimen (Cohort 4)
- ✓ Clinical sites ready to enroll and dose
- ✓ High inbound patient interest based on favorable benefit/risk
- ✓ Patients identified to support completion of clinical study
- ✓ Clear path to BLA submission to support accelerated approval

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 from baseline) and reductions in Left Ventricular Mass (LVMI; ≥10% ↓) at 12-month post-infusion

Cohort 4

Pediatric and Adult/Adolescent (n=12)
Recalibrated dose: 3.8 x 10^{13} GC/kg RP-A501
Revised IM regimen: Rituximab + Steroid + Sirolimus

Patient 7* Patient 8* Patient 9*

+ 9 patients (total n=12)

*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.

Cohort 4 Updates and Rationale

- Dose recalibrated to account for the higher full-to-empty capsid ratio
- Immunomodulation revised based on early Phase 2 safety findings

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;

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

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Preliminary Efficacy of Phase 2 Study RP-A501: Up to 36 months

Preliminary findings show stabilization or improvement in key disease measures

Cohort	Patient	Age at Most RV (y)	Most Recent Visit (m)	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 to RV
Cohort 1: Adult/ Adolescent	2-1	20	24	-36%, 65.5 to 41.9	-28%, 21.8 to 15.6	-7%, 16.5 to 15.4	-19%, 125 to 101	-49%, 0.04 to 0.02	II to I	0, 100 to 100
Cohort 2: Pediatric	2-2	16	36	-35%, 65.4 to 42.8	-39%, 16.0 to 9.7	-10%, 10.4 to 9.4	-7%, 54 to 50	-14%, 0.02 to 0.01	II to I	+2, 98 to 100
	2-3	15	24	+9%, 187.1 to 204.8	-4%, 28.2 to 27.1	-17%, 26.1 to 21.7	-19%, 7966 to 6465	+122%, 0.33 to 0.73	III to I	+16, 64 to 79
	2-4	12	18	-30%, 202.2 to 140.5	+17%, 39.3 to 46.0	+24%, 22.5 to 27.9	-30%, 69855 to 49135	-14%, 0.82 to 0.71	II to II	+35, 35 to 71

All measurements of cardiac structure and function (LVEF, LV Mass, LVMI, IVSd and LVPWd) utilized centrally evaluated echocardiogram data (blinded review) for all participants.
In Phase 2 Cohort 3, two patients treated with immunomodulatory regimen containing a C3-inhibitor experienced serious adverse events.
Cohort 3 is discontinued and data from the two subjects were not included.

Improved Stabilized Worsened

BL, Baseline; cTnI, cardiac troponin I; LAMP2, lysosome-associated membrane protein 2; IVSd, Interventricular 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); NT-Pro-BNP, N-terminal pro–B-type natriuretic peptide; NYHA, New York Heart Association, RV, (Most) Recent Visit; y, year(s)

Data Extraction Date: August 7, 2026

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Modified Protocol Delivers Favorable Pivotal Cohort 4 Safety Profile

Grade ≥3 Serious Treatment Emergent Adverse Events Related to RP-A501

Preferred Term (Grade ≥3)	Phase 2		
	Cohorts 1 & 2 (n=4)	Cohort 3 (n=2)	Cohort 4 (n=3)
Subject with at least 1 Serious TEAE (Grade ≥3)	**3 (75.0)**	**2 (100)**	**1 (33.3)**
Thrombotic microangiopathy [a]	2 (50.0)	1 (50.0)	0
Acute kidney injury	1 (25.0)	1 (50.0)	0
Renal failure	1 (25.0)	0	0
Gamma-glutamyltransferase increased [b]	0	0	1 (33.3)
Hepatic enzyme increased	1 (25.0)	0	0
Cardiac arrest	1 (25.0)	0	0
Posterior reversible encephalopathy syndrome	1 (25.0)	0	0
Rhabdomyolysis	1 (25.0)	0	0
Aplastic anemia	0	1 (50.0)	0
Disseminated intravascular coagulation	0	1 (50.0)	0
Cytokine release syndrome	0	1 (50.0)	0
Hypertransaminasaemia	0	1 (50.0)	0
Pneumothorax	0	1 (50.0)	0
Sepsis	0	1 (50.0)	0
Shock	0	1 (50.0)	0
Retroperitoneal hemorrhage	0	1 (50.0)	0

Pivotal Cohort 4
(recalibrated dose with modified IM regimen)

- No evidence of thrombotic microangiopathy, capillary leak or other complement-mediated events
- Elevated GGT observed in 1 patient; resolved and patient discharged after 12 days.
- All 3 patients discharged by 1-2 weeks post-infusion

[a]Patients 2-3, 2-4 and 2-6; [b]Patient 2-8.
GGT, Gamma-glutamyltransferase; IM, immunomodulatory; TEAE, Treatment Emergent Adverse Events
Data Extraction Date: Aug 7, 2026

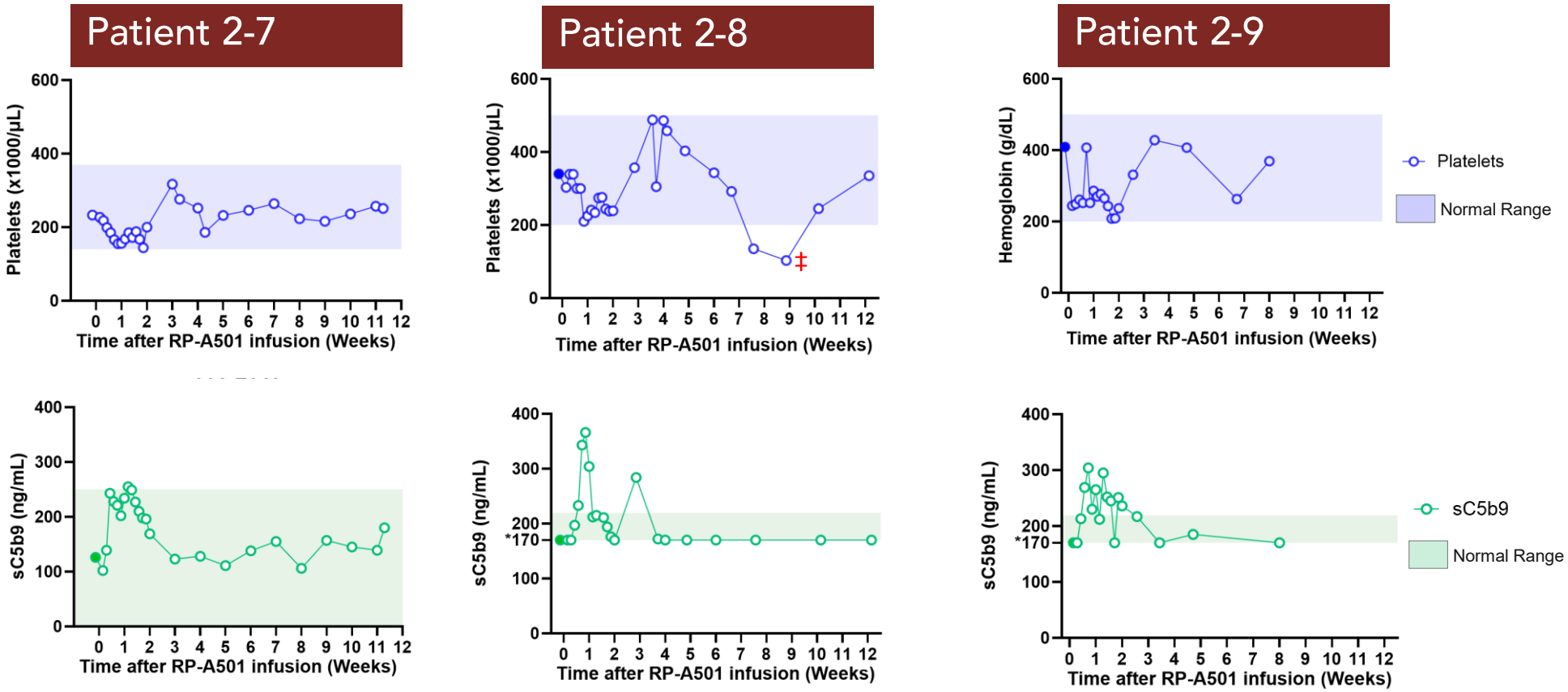
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No Evidence of TMA, CLS, or Other Severe Complement-Mediated Toxicities in Cohort 4

Platelets and Terminal Complement Complex (sC5b9) of the initial 3 patients up to 3 months post-treatment

Platelets

Complement Marker (sC5b9)



‡ A decrease in platelet count was observed at Week 8-9 in the absence of symptoms or signs suggestive of platelet dysfunction or deficit. All concomitant medications were reviewed to discontinue any potential contributors; it was not deemed clinically significant by the investigator and Sponsor and resolved following medication modifications.

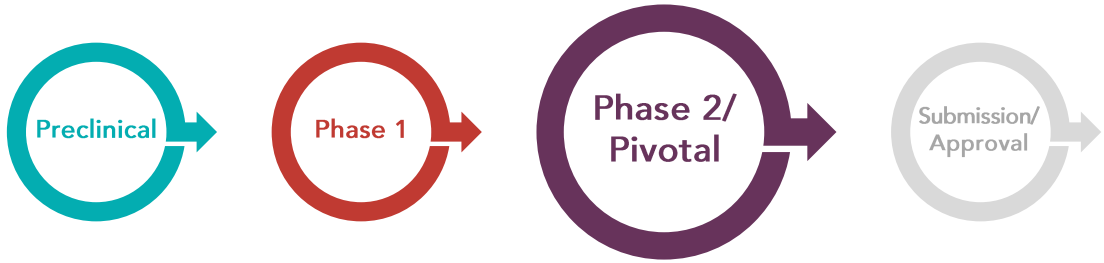
*The sC5b9 lower limit of detection per the validated assay at the clinical site is <170 ng/mL; reported values of <170 ng/mL are shown as 170 ng/mL.

CLS, Capillary Leak Syndrome; sC5b9, soluble C5b9; TMA, Thrombotic microangiopathy

Data Extraction Date: September 4, 2026

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Pivotal Phase 2 Trial of RP-A501 is Ongoing



Phase 1 Safety & Efficacy

- Phase 1 clinical benefit observed up to 7 years, with durable biomarkers at latest follow-up
- 100% overall survival at median 5.7-year follow-up (range: 4.3–7.1 years); Median transplant-free survival not reached

Pivotal Phase 2 trial

- Early Phase 2 results mirror Phase 1, with improvement or stabilization in cardiac hypertrophy, heart failure symptoms, quality of life and cardiac biomarkers
- Enrollment and dosing ongoing for 9 additional patients in Cohort 4

NEXT STEPS

- Dosing completion targeted for mid-2027
- Topline 12-month data expected in 2H 2028

Regulatory Designations

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

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

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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

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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

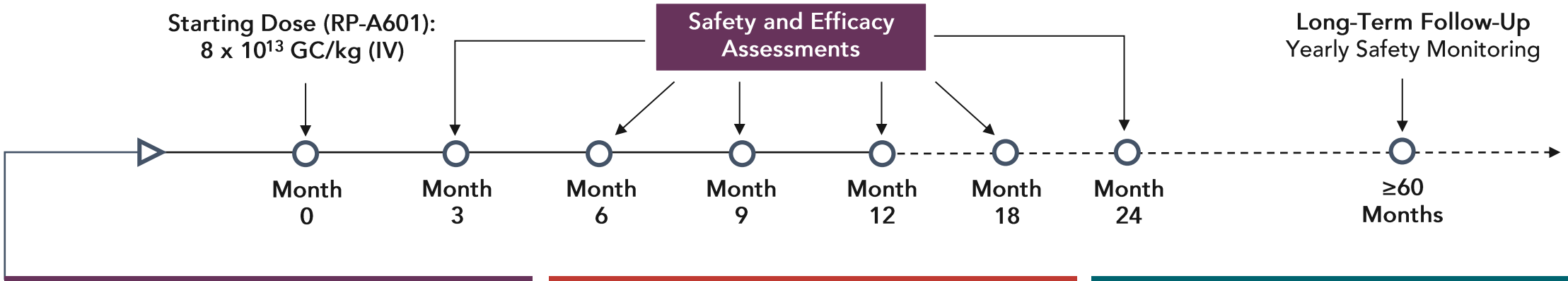
ACM, arrhythmogenic cardiomyopathy; PKP2, plakophilin 2

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

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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

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Cardiovascular Health is Dependent on Functional BAG3 Protein

BAG3-Dilated Cardiomyopathy (BAG3-DCM)

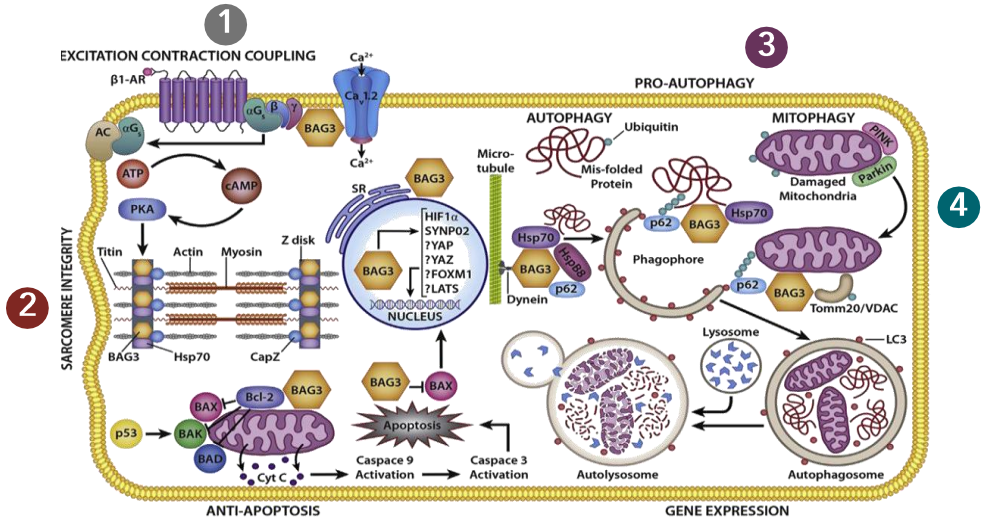


- 20%-50% of DCM patients have familial DCM; up to 40% of whom have an identifiable genetic cause[3,4]
- 80% of patients over 40 years of age with a BAG3 mutation will present with the disease[2]
- Pathogenic variants in BAG3 are estimated to cause 2.3%-6.7% of DCM cases in the US, Europe, & Japan[2]

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.

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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

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

Restoration of Cardiac Protein Expression

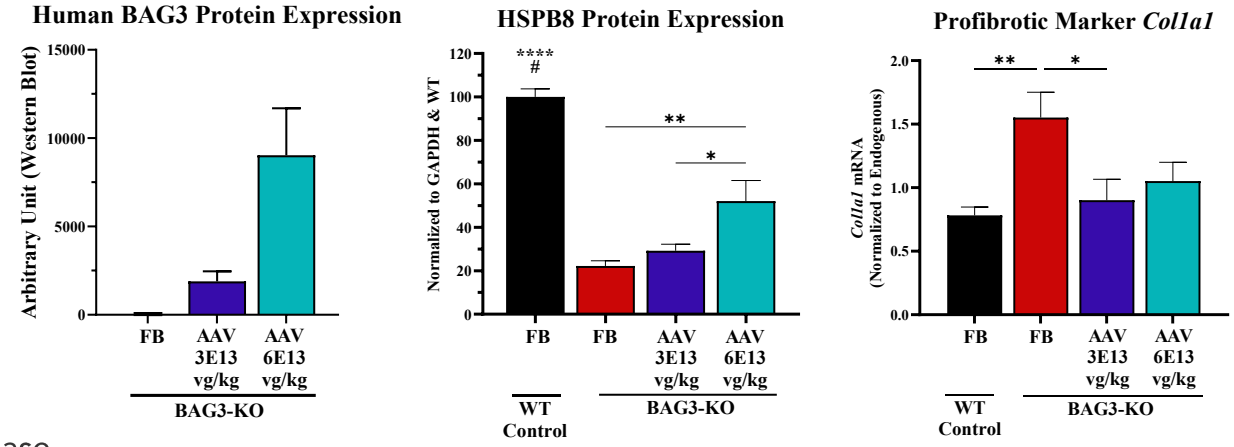
- AAVrh.74-BAG3 produced dose-dependent changes in cardiac protein expression, beyond the BAG3 protein including:
  - Dose-related increase in myocardial HSPB8, a key BAG3 co-chaperone
  - Robust reduction in myocardial expression of the profibrotic marker *Col1a1*

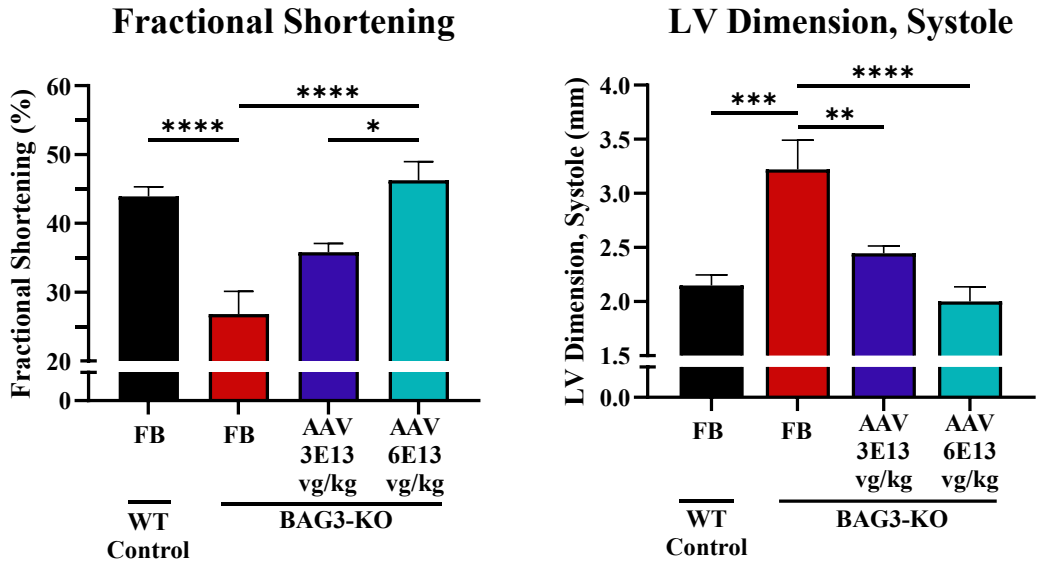
Dose-Dependent Improvement of Cardiac Function

- 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





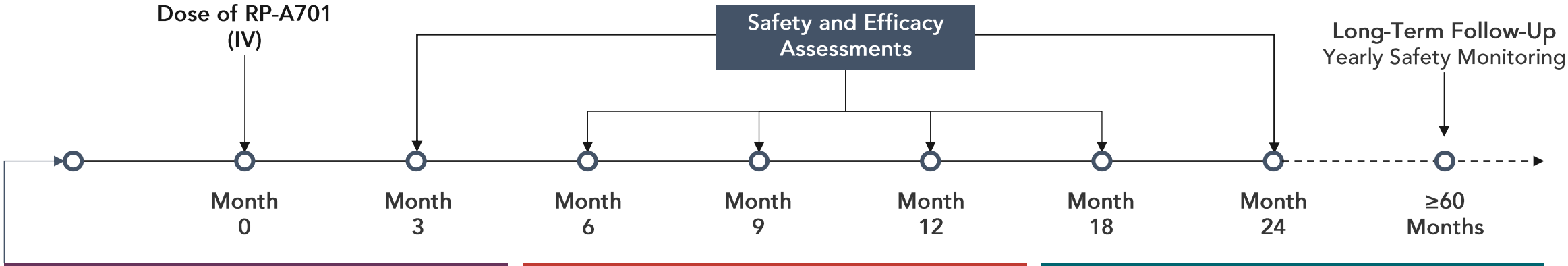
RP-A701 (AAVrh.74-BAG3) is an AAVrh.74-based gene therapy encoding human BAG3 under the control of a cardiac-selective promoter. All nonclinical studies shown used a toxicology lot manufactured using a process representative of the clinical manufacturing process.
FB, Formulation Buffer; LV, left ventricle; WT, Wild Type

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

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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: Dosing initiated in the Phase 1 trial of RP-A701 for BAG3-DCM.

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

Market Opportunity[1-3] – US Annual incidence of ~25 individuals

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

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

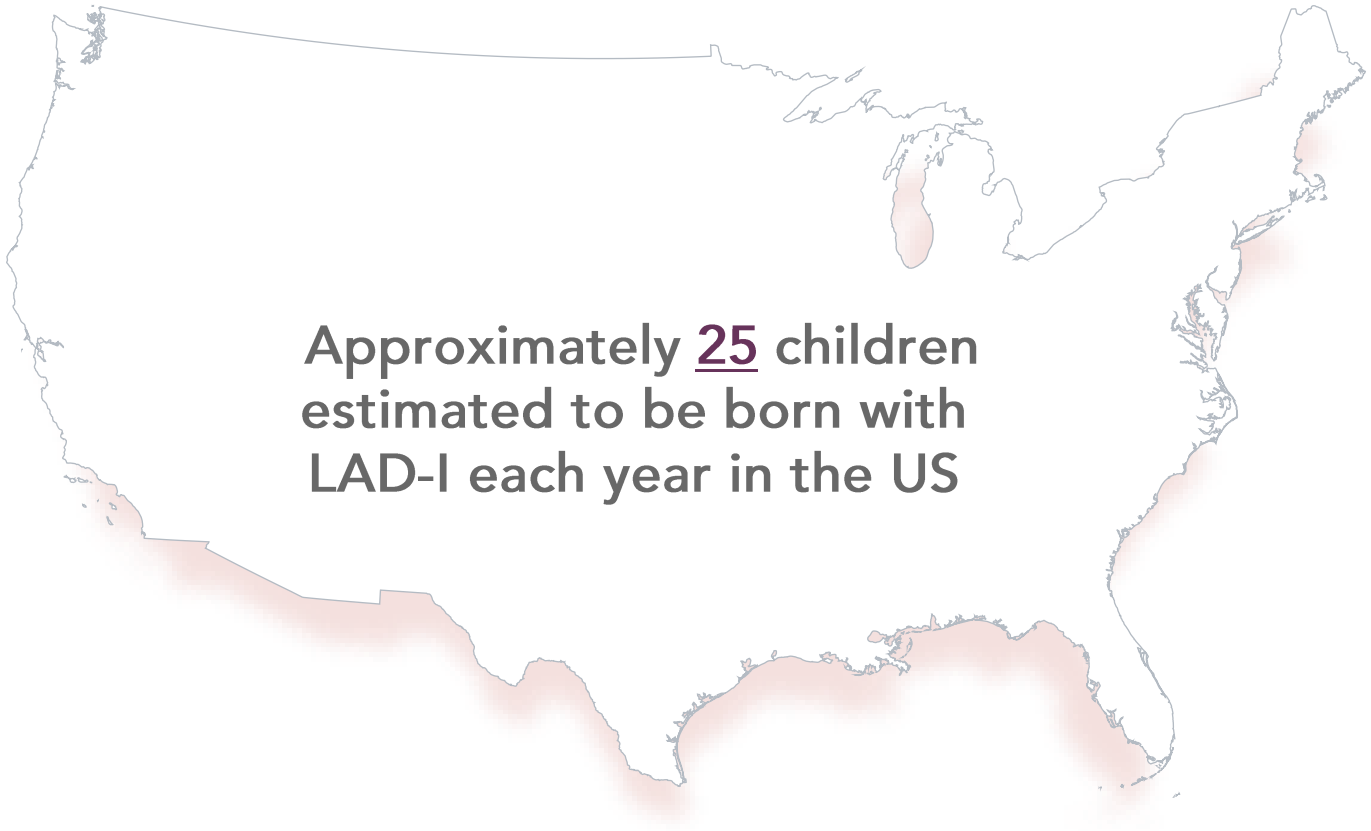
KRESLADI™

marnetegragene autotemcel

rocket pharma

*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).

KRESLADI™ Ready for First Commercial Patient Enrollment



Commercial Patient Enrollment Now Open

- Rocket achieved commercial readiness for KRESLADI™ on October 1, 2026.
- Two Qualified Treatment Centers (QTCs) are activated, and Rocket is ready to begin patient enrollment.
- The commercialization strategy remains centered on a limited network of specialized QTCs and an appropriately scaled commercial infrastructure, supporting patient access while maintaining disciplined investment.

LAD-I, leukocyte adhesion deficiency-I.

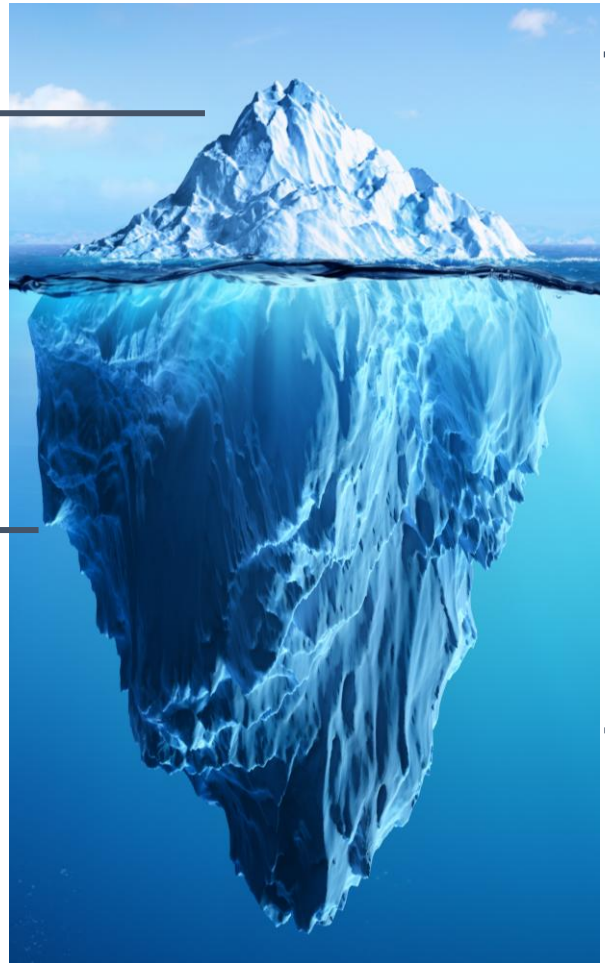
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Wave 2: Future Cardiovascular Gene Therapies

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



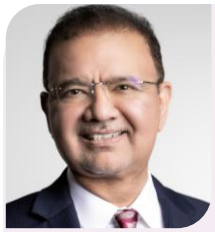
Gaurav Shah, M.D.
Chief Executive Officer
Spearheaded Kymriah development at Novartis towards approval.

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



Sarbani Chaudhuri
Chief Commercial 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 BIOSCIENCE CORPORATION

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