

RP-A501 Danon Disease Program Update

Rocket Pharmaceuticals

October 6, 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 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.

RP-A501 Danon Disease Program Update

Compelling development path: \$1B+ commercial opportunity

Updated Phase 1 data

Evidence of disease reversal or stabilization up to 7 years post RP-A501

Initial Phase 2 data

Preliminary efficacy up to 36 months data from Cohorts 1-3

Recalibrated dose safety

Positive safety observations: 3 patients treated safely at recalibrated dose + optimized immunomodulation

Pivotal path

FDA agreement to complete Cohort 4 supporting BLA submission for accelerated approval; PRV eligible

Commercial Opportunity

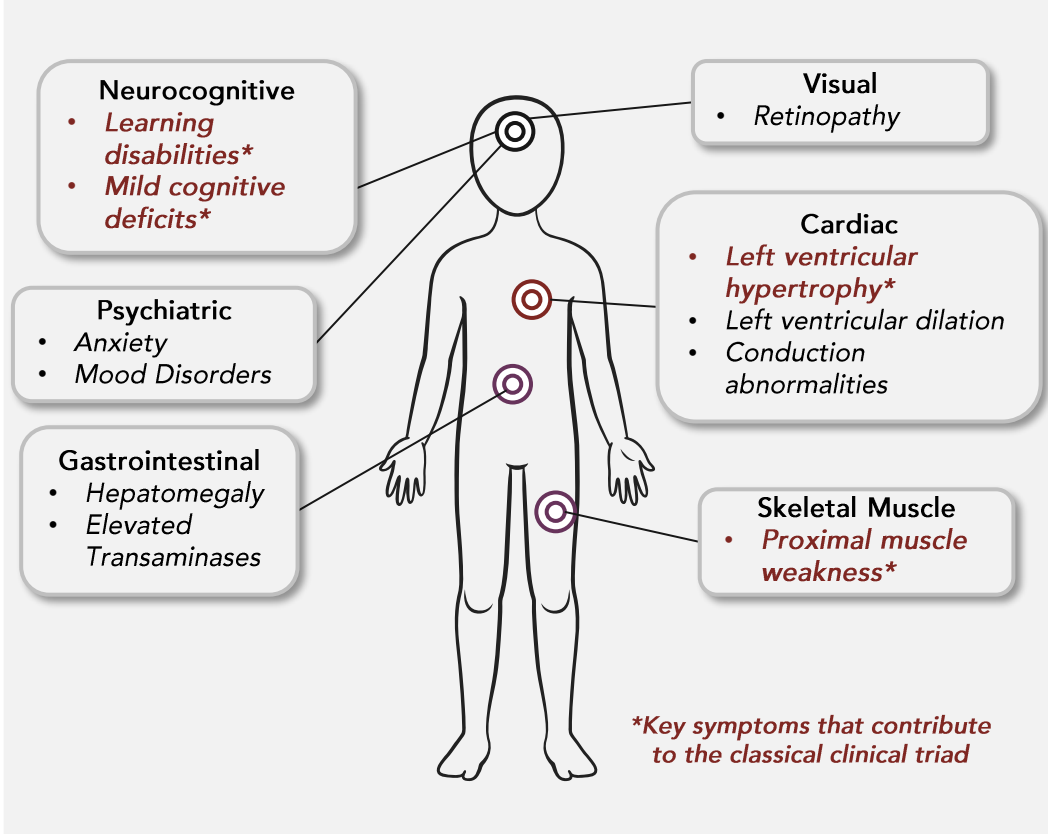
10-11K US epidemiology (**20K+** including EU5); **900+** Danon patients recorded in US; **\$1B+** global peak opportunity with initial indication

Danon Disease is a Highly Aggressive, Underdiagnosed, Rare Genetic Cardiomyopathy

Catastrophic in males, with rapid progression and early mortality

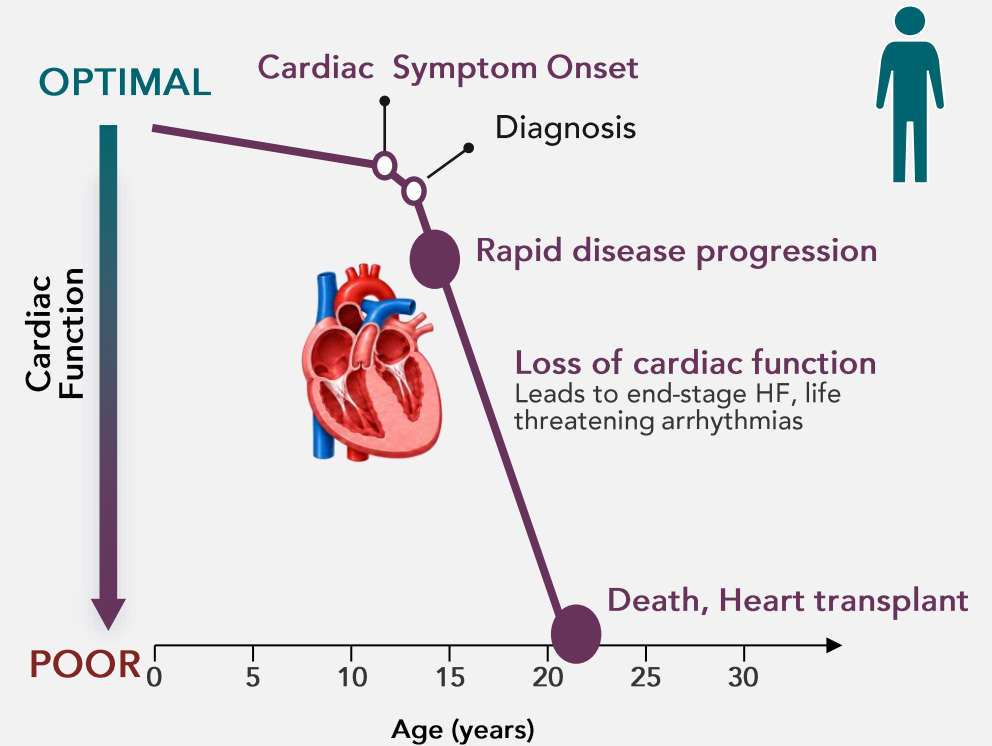
Clinical Presentation

Hallmark Cardiac Manifestation and Potential Multi-System Involvement¹



Danon Males

Rapid Decline from Symptom Onset to End-Stage Heart Failure²⁻⁴



Illustrative; derived from natural history registries & cohorts

No Approved Disease-Modifying Therapy Exists

Heart transplantation is the only end-stage rescue and only for a few



Only ~25% ultimately receive heart transplant (HTx)

- Donor scarcity & matching constraints
- Diagnosis, referral & listing delays
- Clinical deterioration/eligibility loss

HTx is lifesaving, but carries early mortality risk

HTx can be effective

10+ yrs Median survival after HTx in general population

87.1% 5-year graft survival in Danon HTx cohort

Early mortality remains

5 of 8 Died within 1 year after HTx/LVAD in a Danon cohort

HTx carries substantial lifelong medical and family burden

- Lifelong immunosuppression
- Rejection & serious infection
- Organ toxicity
- Graft failure/retransplantation



“ I’d lost both my mum and brother as they waited for transplants.”

Danon patient and emergency HTx recipient; daughter also has Danon - 4 affected family members across 3 generations.

“ I thought it would be for a couple days. But that turned into eight months.”

Mother of Danon patient - deterioration and mechanical support while awaiting a donor heart

Transplant-Free Survival Matters: More Time Alive with Own Functioning Heart

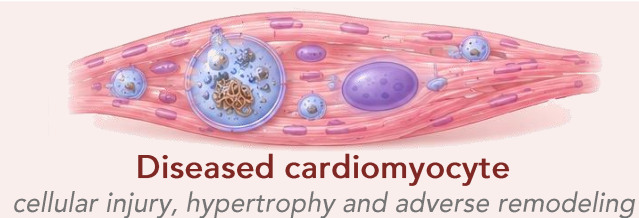
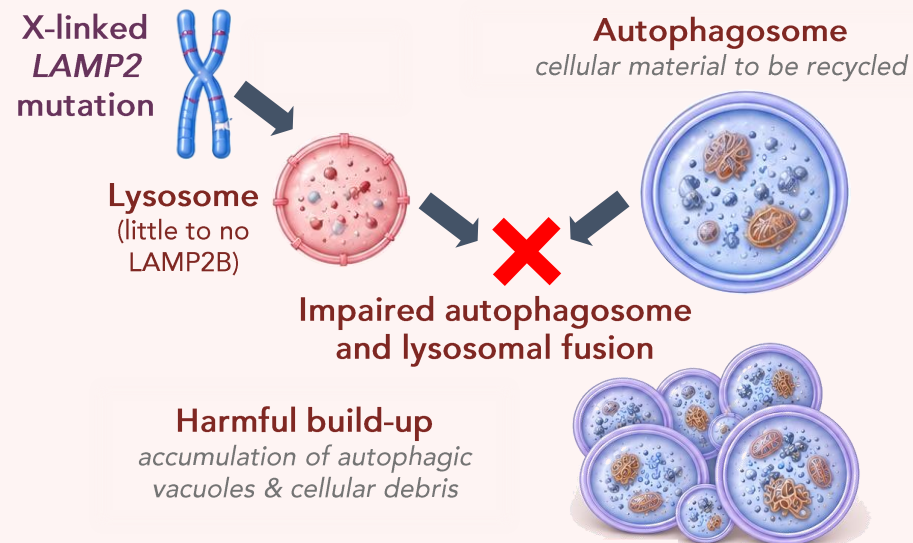


HTx, heart transplantation; LVAD, left ventricular assist device;
1. Hong et al. JAHA. 2025;14:e038394. 2. ISHLT Fast Facts. 2024. 3. Hong et al. J Card Fail. 2022;28:664–669. 4. Lotan et al. Circ Genom Precis Med. 2020;13:e003117. 5. Velleca et al. J Heart Lung Transplant. 2023;42:e1–e141.
6. Royal Brompton & Harefield Hospitals, “My story — Caroline Earnshaw”: <https://www.rbht.nhs.uk/patients-visitors/patients/patient-support-services/patient-experience-and-involvement/patient/my-story-caroline-earnshaw>.
7. Cleveland Clinic Children’s, “Heart Transplant Patient Finds New Life at Cleveland Clinic Children’s”: <https://my.clevelandclinic.org/patient-stories/100-heart-transplant-patient-finds-new-life-at-cleveland-clinic-childrens>.

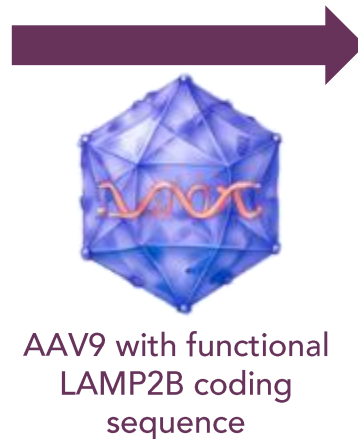
Danon Disease Biology is Exceptionally Suited to LAMP2B Gene Addition

A clear molecular defect. A direct therapeutic approach. A measurable path from biology to clinical impact.

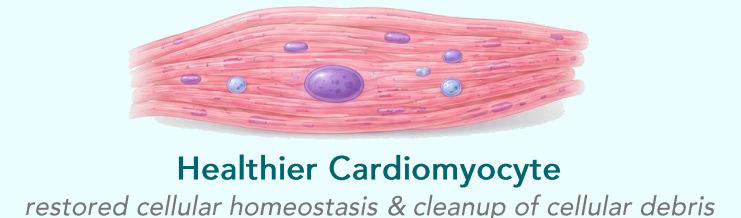
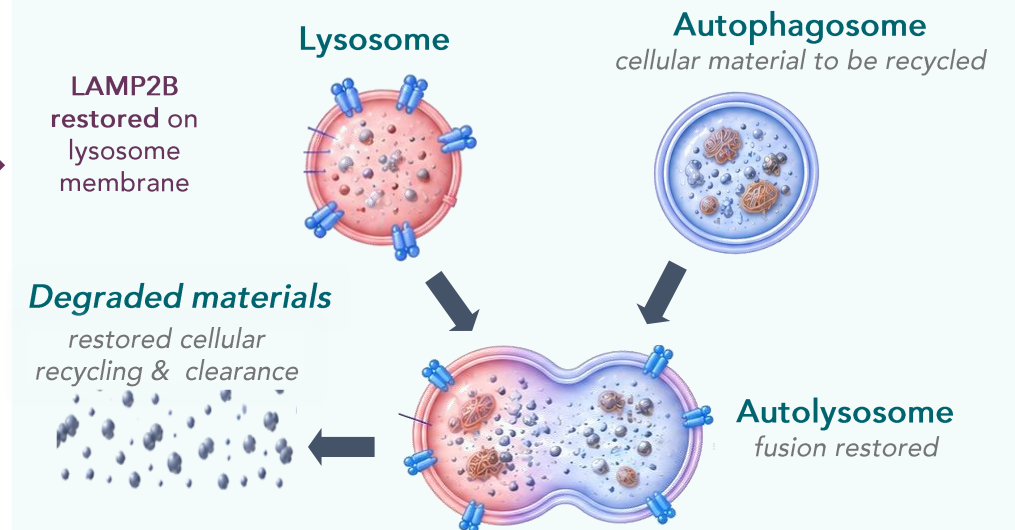
DANON DISEASE: *LAMP2B* deficiency



Gene addition
to
cardiomyocytes



LAMP2B Gene Addition: Restores Cellular Recycling



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 '24¹

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 <i>+Included initial sequential Pediatric Safety Run-in (n=2)</i>
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

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%)

Cohort 4	Adult/Adolescent and Pediatric (n=12) 3.8 x 10 ¹³ GC/kg RP-A501 IM regimen: Rituximab + Steroid + Sirolimus
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← **Pivotal Phase 2 Trial**



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

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
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	++	NP
6.7 × 10 ¹³ GC/kg Pediatric	6	0	++	+	NP	++	NP	++	
	7	0	+	++	NP	++	NP	++	

Legend: IHC Staining Grade¹ (% Positive Cardiomyocytes)

Grade 0 = no staining + = Grade 1 (≤25%) ++ = Grade 3 (51%–74%)

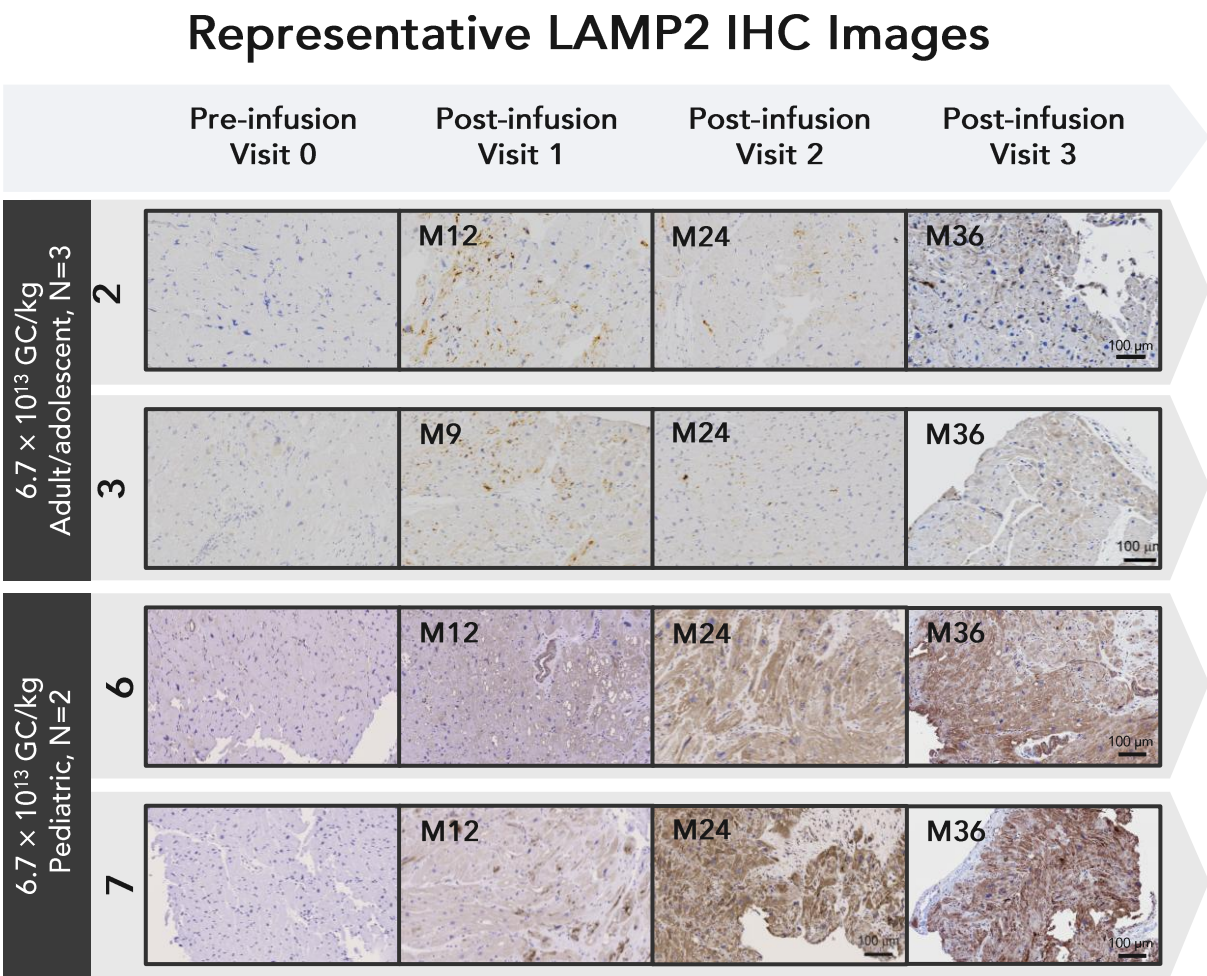
NP = not performed ++ = Grade 2 (26%–50%) +++ = Grade 4 (≥75%)

†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

¹Grading 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.



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.

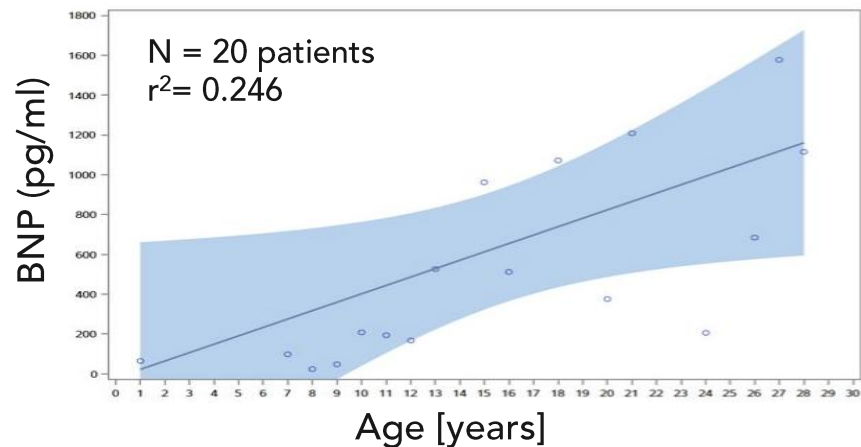
^aPatient underwent heart transplant at 4.8 years post-RP-A501 infusion; post data cut.

Improved Stabilized Worsened

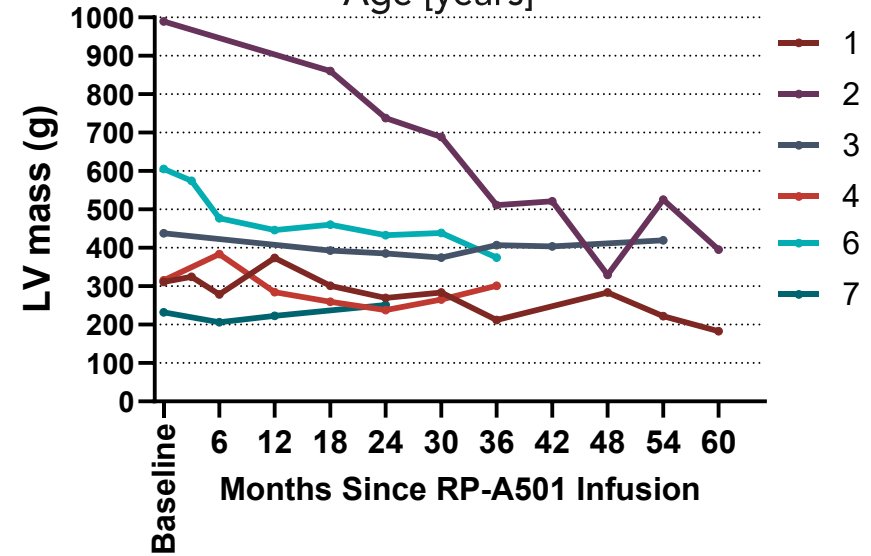
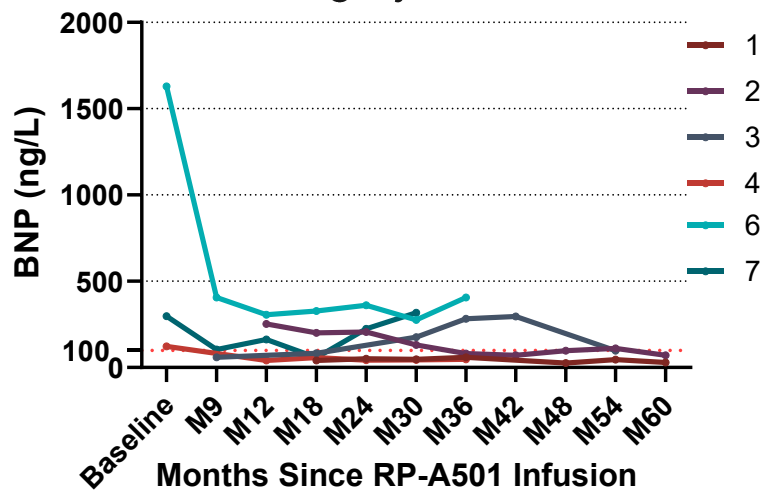
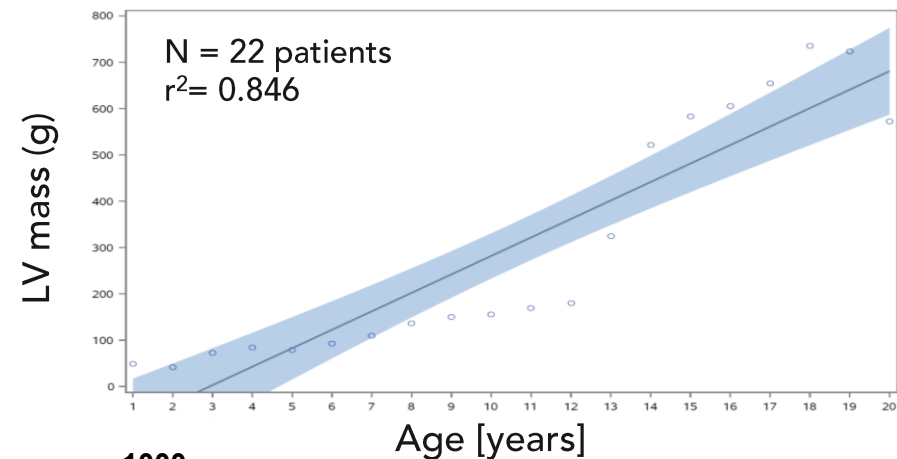
Improved or Stabilized Cardiac Biomarkers and LV mass in RP-A501 Patients

Differs significantly from expanded natural history data

BNP increases by 42.17 ± 18.46 pg/mL per year in untreated Danon males*



LV Mass increases by 39.86 ± 4.01 g per year in untreated Danon males*



*Unpublished data from International Danon Disease Registry
BNP, Brain Natriuretic Peptide; LV, Left Ventricle; Data Extraction Date: July 31, 2026 (Phase 1 RP-A501 study)

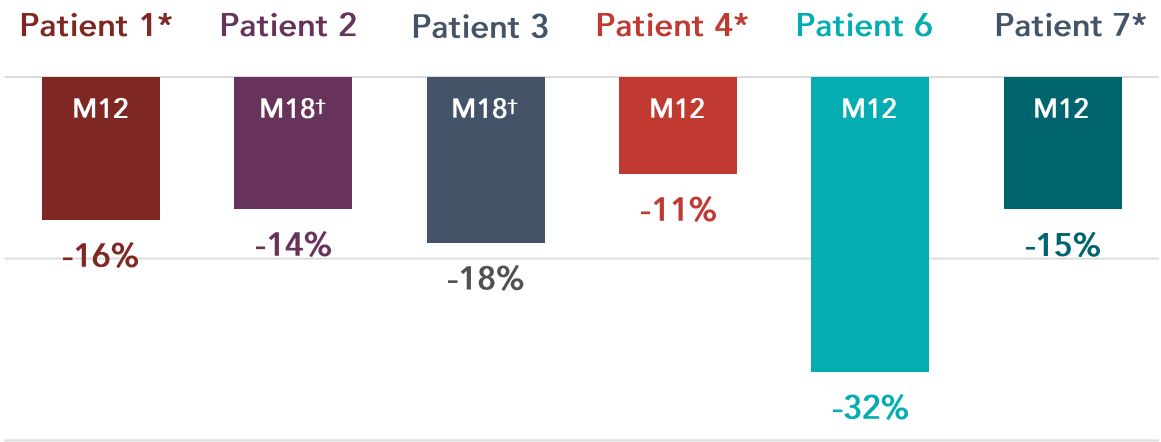
RP-A501 Phase 1 Summary

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

RP-A501 Pivotal Phase 2 Trial Design

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

- ✓ 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 ($\geq$ Grade 1 from baseline) and reductions in Left Ventricular Mass (LVMI; $\geq 10\%$ ↓) at 12-month post-infusion

Cohort 4

Pediatric and Adult/Adolescent (n=12)

Recalibrated dose: 3.8×10^{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.

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

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



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

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

^aPatients 2-3, 2-4 and 2-6; ^bPatient 2-8.



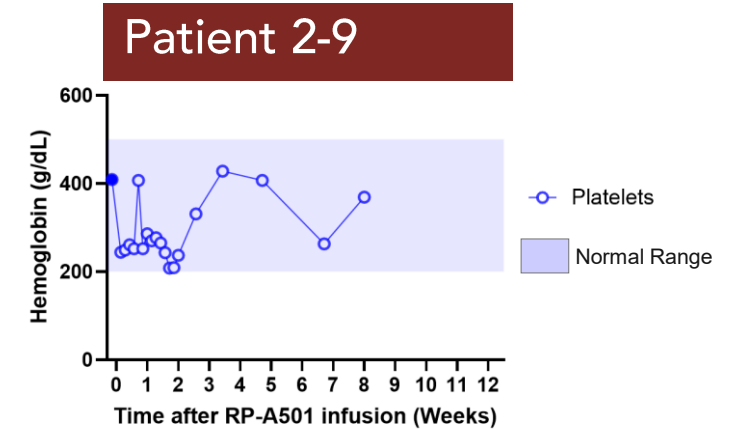
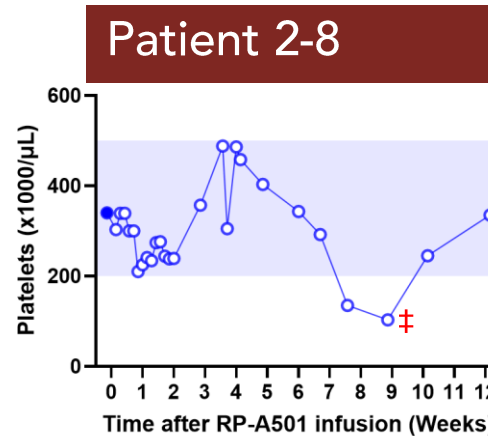
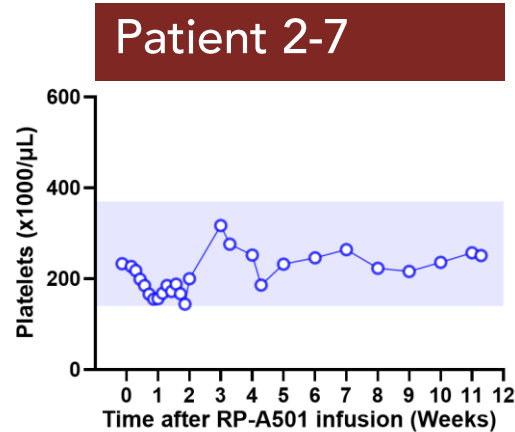
GGT, Gamma-glutamyltransferase; IM, immunomodulatory; TEAE, Treatment Emergent Adverse Events

Data Extraction Date: Aug 7, 2026

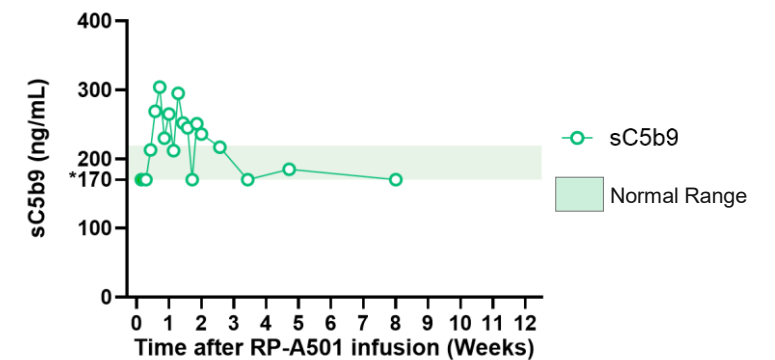
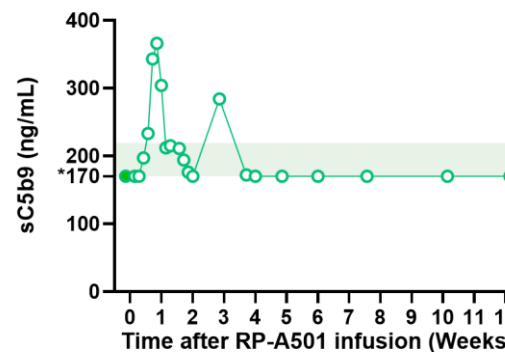
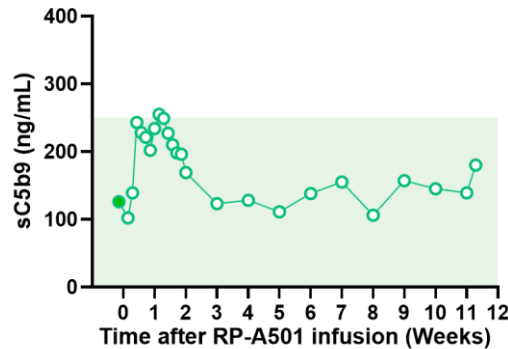
No Evidence of TMA, CLS, or Other Severe Complement-Mediated Toxicities in Cohort 4

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.

RP-A501 Demonstrated Durable Efficacy with a Tolerable Safety Profile

PHASE 1 DURABILITY

Up to 7 Years of clinical benefit observed

Clinical benefit and improvements observed with durable biomarker in all patients at latest trial follow-up

100% overall survival; median transplant-free survival not reached at median follow-up: 5.7 years (range: 4.3–7.1 years)

PHASE 2 EARLY EFFICACY

Mirrors Phase 1 Trajectory

Improvement or stabilization of cardiac hypertrophy, heart failure symptoms, quality of life scores, and biomarkers of cardiac injury and stress replicate the trajectory of Phase 1 responders

SAFETY AND TOLERABILITY

Generally Well-Tolerated

Recent protocol optimizations suggest successful mitigation of complement-mediated toxicities*

RP-A501 Pivotal Phase 2 Milestones

TODAY

Enrollment
and dosing

9 additional patients
in Cohort 4

MID-2027

Dosing
completion

Last patient dosed
12-month follow-up

MID-2028

TOPLINE
TARGET*

Primary efficacy
readout from pivotal
Cohort 4

LATE-2028

BLA regulatory
filing

PRV eligible;
Accelerated Approval;
Priority review

CO-PRIMARY EFFICACY ENDPOINTS AT 12 MONTHS

$\geq$ Grade 1

Improvement in LAMP2
protein expression from
baseline

$\geq 10\%$

Reduction in left ventricular
mass index (LVMI)



*MID-2028 target is provisional pending alignment with dosing completion and 12-month follow-up.

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 categories¹ from gnomAD²

38%
(~4.1K)

Scaling

Other P/LP *LAMP2* variants

6 variant categories³ identified through RW datasets⁴ 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

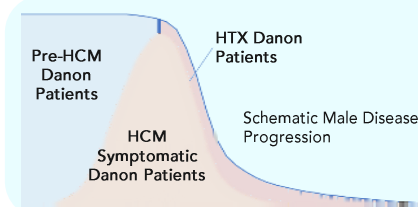
Patients with HCM Phenotype⁵

Age & sex-specific DD yield modeling with HCM population

~5.4K

Scaling

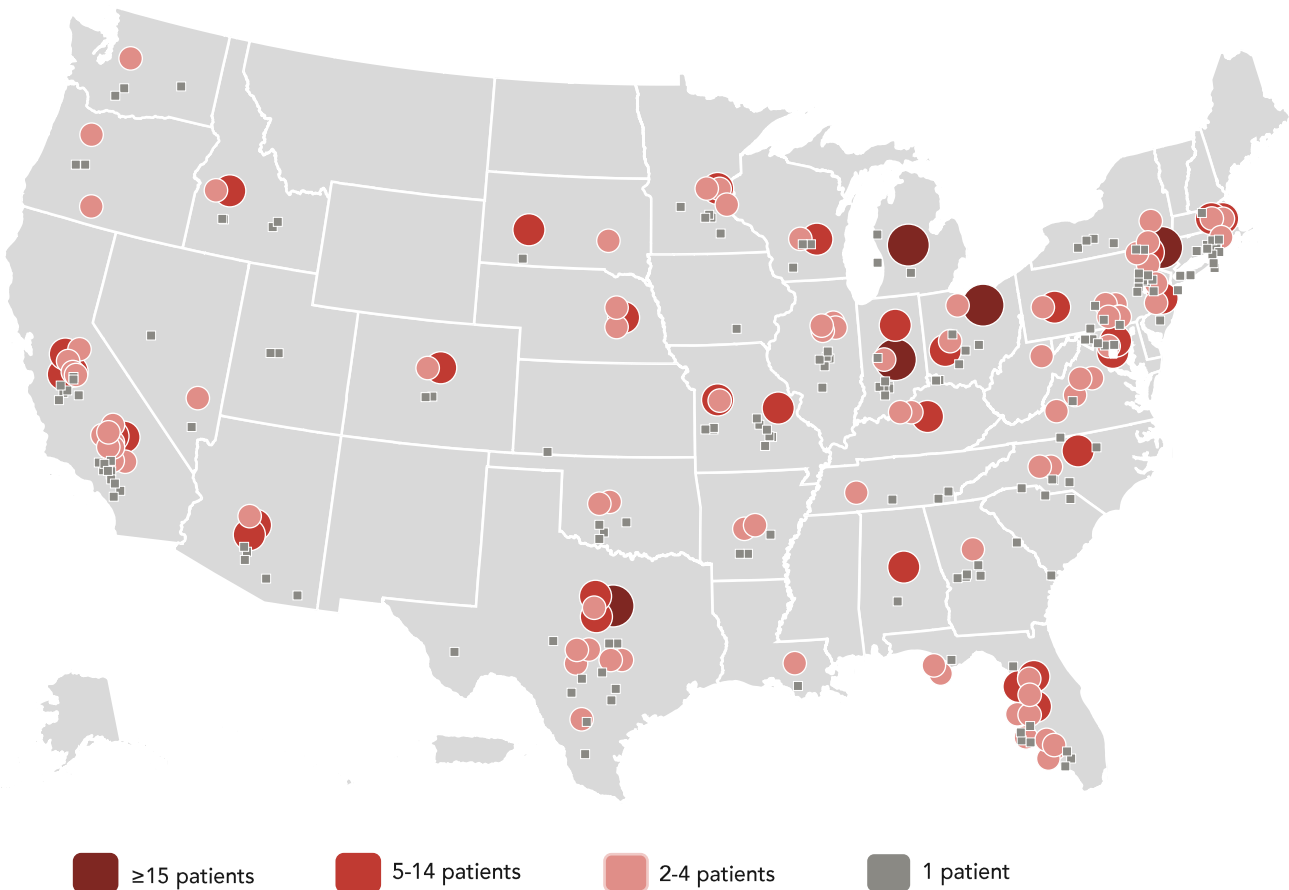
Pre-cardiomyopathy and DCM Patients



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

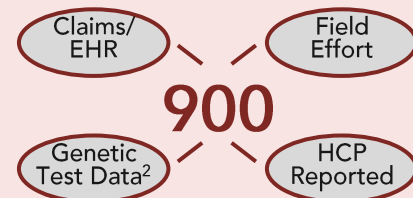
Over 900 Identified US Danon Disease Patients

Representing a current diagnosis rate under 10%



Patients cumulatively identified and include those who received HTx or subsequently experienced disease-related mortality

Sources¹



De-duplicated for unique³ patient counts

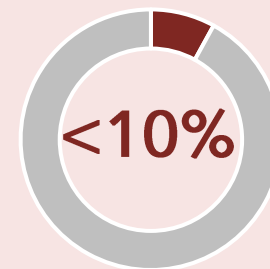
Institutional coverage (% of pts)



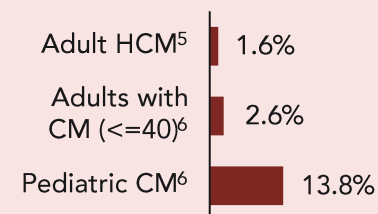
Gender



Est. DD Diagnosis Rate⁴



Genetic Testing Rates in Cardiomyopathies (%)



Accelerating Danon Diagnosis: Focused Path to Launch

Aspiration: every person with HCM or a Danon disease signature and every at-risk relative of a confirmed Danon patient is genetically evaluated so no Danon diagnosis is missed

Drive cardiologists to test young HCM & urgency with Danon signature



Expand diagnostic infrastructure & access



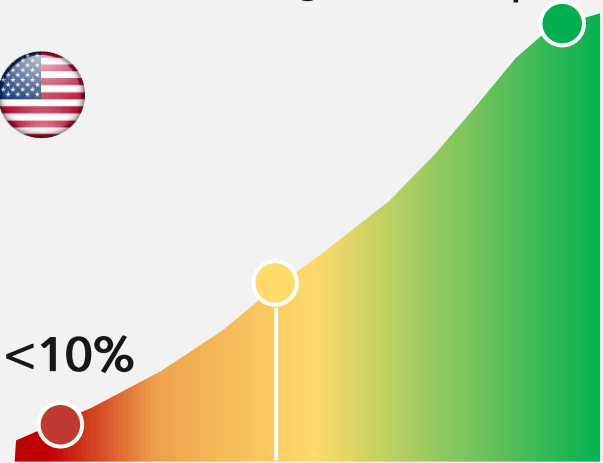
Empower patients & caregivers to seek genetic evaluation



Multiply diagnoses through the family cascade



Illustrative Diagnoses Ramp



At launch
(Mid-2029)

Longer term

Diagnosed Share of Target Population

External
tailwinds



↑ HCM recognition & CMI entry into pediatrics



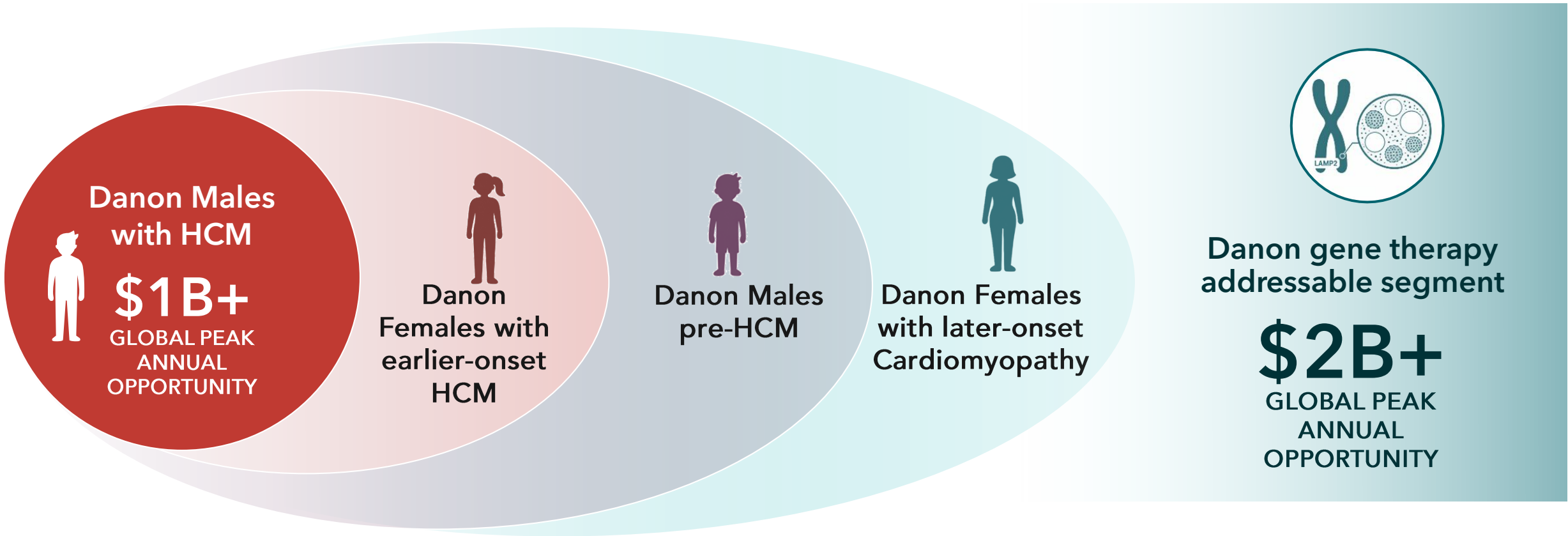
↑ Genotype-driven trials in cardiomyopathies



↑ Ecosystem momentum around cardio genomics & broader testing

Addressable Opportunity in Danon Extends Beyond Males with HCM

Expand the window, extend the impact, transform the disease



UNLOCK DANON OPPORTUNITY

Treat earlier
Shift treatment upstream

Capture fast progressors
Intervene before irreversible progression

Reach underdiagnosed females
Treat patients historically missed

New Evidence and a Defined Pivotal Path

Sustained Clinical Benefit

RP-A501 conferred durable disease reversal/stabilization through 36 months and up to 7 years post-RP-A501 infusion

Preliminary efficacy in initial 4 patients in Phase 2 further corroborates long-term benefit identified in Phase 1 and LTFU

Early safety at the recalibrated dose

No TMA, capillary leak, or significant complement-mediated adverse events through 3 months in the first three patients

Attractive Commercial Opportunity

US epidemiology of 10-11K US (20K including Europe); over 900 Danon disease patients recorded in US alone; \$1B global peak potential for 1st indication

Efficacy target

At least

7 of 12

responders at 12 months

Each responder must meet both criteria

Myocardial LAMP2 expression $\geq$ Grade 1 from baseline

LVMI reduction $\geq$ 10% from baseline

Dosing completion expected mid-2027

Current cash runway now into Q3'28

