UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, DC 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 OR 15(d) of The Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): September 12, 2023

Rocket Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**001-36829**	**04-3475813**
(State or other jurisdiction of incorporation)	(Commission File Number)	(IRS Employer Identification No.)

9 Cedarbrook Drive, Cranbury, NJ	**08512**
(Address of principal executive offices)	(Zip Code)

Registrant's telephone number, including area code: (646) 440-9100

Not applicable
(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common stock, $0.01 par value	RCKT	The Nasdaq Global Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 7.01 Regulation FD Disclosure.

Attached as Exhibit 99.l to this Current Report on Form 8-K, and incorporated into this Item 7.01 by reference, is an investor presentation (the "Investor Presentation") prepared by Rocket Pharmaceuticals, Inc. (the "Company") providing certain updates on the Company's Danon Disease Program, including the alignment it recently achieved with the Food and Drug Administration (FDA) on the design of the Phase 2 pivotal trial of RP-A501 for Danon Disease.

The information in this Item 7.01, including Exhibit 99.1 is furnished and shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to liabilities under that section, and shall not be deemed to be incorporated by reference into the filings of the Company under the Securities Act of 1933, as amended, or the Exchange Act, regardless of any general incorporation language in such filings. This Current Report on Form 8-K will not be deemed an admission as to the materiality of any information contained in this Item 7.01, including Exhibit 99.1.

Item 8.01. Other Events.

On September 12, 2023, the Company issued a press release announcing that alignment has been reached with the FDA on the design of the Phase 2 pivotal trial of RP-A501 for Danon Disease. A copy of the press release is attached hereto as Exhibit 99.2 to this Current Report on Form 8-K and is incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Investor Presentation dated September 11, 2023.
99.2	Press Release dated September 12, 2023.
Exhibit 104	Cover Page Interactive Data File (embedded within the Inline XBRL document).

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: September 12, 2023

Rocket Pharmaceuticals, Inc.

By: /s/ Gaurav Shah, MD
Name: Gaurav Shah, MD
Title: Chief Executive Officer and Director

Exhibit 99.1


Rocket Pharmaceuticals
Danon Disease Program Update
September 11, 2023
rocket
pharma
SEEKING GENE THERAPY CURES

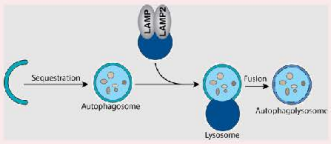
Disclaimer

Various statements in this presentation concerning Rocket's future expectations, plans and prospects, including without limitation, Rocket's expectations regarding the safety and effectiveness of product candidates that Rocket is developing to treat Danon Disease (DD), 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 Danon Disease program, including its planned pivotal trial, and the safety, effectiveness and timing of related pre-clinical studies and clinical trials, may constitute forward-looking statements for the purposes of the safe harbor provisions under the Private Securities Litigation Reform Act of 1995 and other federal securities laws and are subject to substantial risks, uncertainties and assumptions. You should not place reliance on these forward-looking statements, which often include words such as "believe," "expect," "anticipate," "intend," "plan," "will give," "estimate," "seek," "will," "may," "suggest" or similar terms, variations of such terms or the negative of those terms. Although Rocket believes that the expectations reflected in the forward-looking statements are reasonable, Rocket cannot guarantee such outcomes. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including, without limitation, Rocket's ability to monitor the impact of COVID-19 on its business operations and take steps to ensure the safety of patients, families and employees, the interest from patients and families for participation in each of Rocket's ongoing trials, patient enrollment, trial timelines and data readouts, our expectations regarding our drug supply for our ongoing and anticipated trials, actions of regulatory agencies, which may affect the initiation, timing and progress of pre-clinical studies and clinical trials of its product candidates, our ability to submit regulatory filings with the U.S. Food and Drug Administration (FDA) and to obtain and maintain FDA or other regulatory authority approval of our product candidates, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, our competitors' activities, including decisions as to the timing of competing product launches, pricing and discounting, our integration of an acquired business, which involves a number of risks, including the possibility that the integration process could result in the loss of key employees, the disruption of our ongoing business, or inconsistencies in standards, controls, procedures, or policies, our ability to successfully develop and commercialize any technology that we may in-license or products we may acquire and any unexpected expenditures, as well as those risks more fully discussed in the section entitled "Risk Factors" in Rocket's Annual Report on Form 10-K for the year ended December 31, 2022, filed February 28, 2023 with the SEC and subsequent filings with the SEC including our Quarterly Reports on Form 10-Q. Accordingly, you should not place undue reliance on these forward-looking statements. All such statements speak only as of the date made, and Rocket undertakes no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

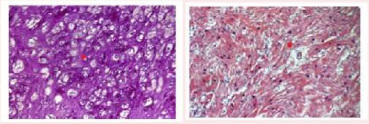
Danon Disease

Aggressive genetic hypertrophic cardiomyopathy with very high early mortality

1 **Gene mutation leads to decreased LAMP2B protein and impaired autophagy**



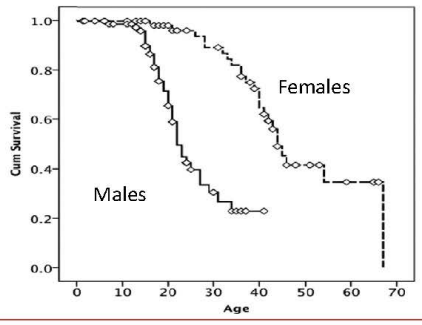
2 **Impaired autophagy leads to ↑ vacuoles, myocyte hypertrophy, necrosis, & fibrosis**



3 **Pathologic processes lead to phenotype of extreme LV hypertrophy and cardiomyopathy**

- Danon Disease is the most aggressive and lethal hypertrophic cardiomyopathy
- Monogenic, X-linked disease progresses to severe cardiomyopathy at early ages; characterized by massive LV hypertrophy and end-stage heart failure
- Uniformly fatal with early mortality (*males ~19 yrs.; females ~37 yrs.)*
- Only definitive treatment is cardiac transplantation, availability is limited, and associated with extensive short- and long-term morbidity and mortality (<50% 10-yr survival post HTx)
- Estimated prevalence of 15,000 to 30,000 individuals and annual incidence of 800 to 1,200 individuals in US & EU
- In July 2023, US Dept. Health and Human Services approved IDC 10 Code for DD (E74.05)

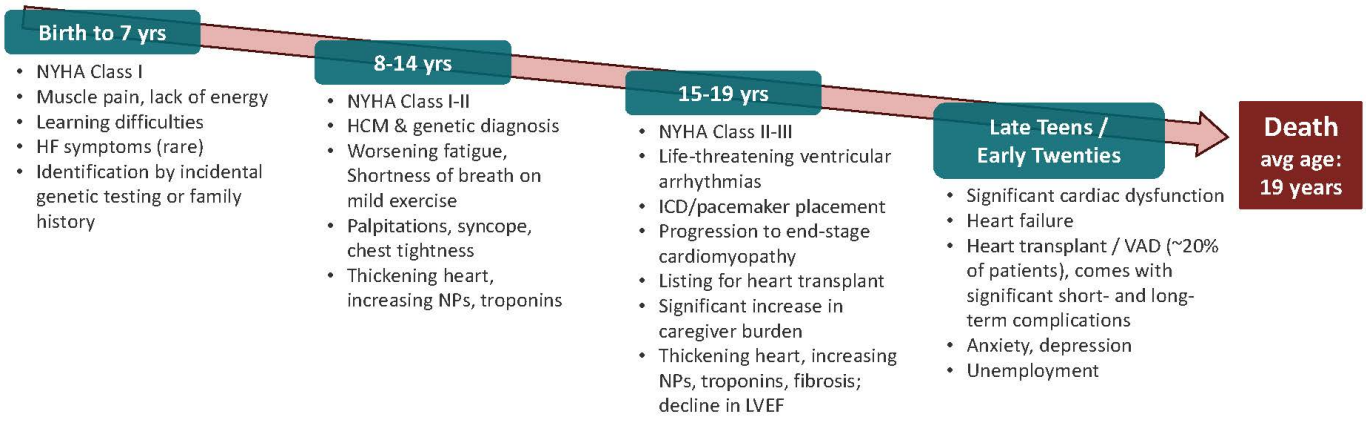
4 **Natural History: High rates of death at early ages**



Cenacchi G et al. Neuropathol. Appl. Neurobiol. 2020; Rowland T et al. J Cell Sci. 2016; Boucek D, Jirikowic J, Taylor M. Genet Med 2011; Thrush P et al. J Thorac Dis. 2014; Dipchand A et al. J Heart Lung Transplant. 2014

Natural History: Progression of Danon Disease in Male Patients

Critical interval in childhood / early adolescence precedes rapid decline, providing optimal window of opportunity for GTx
Patients typically on maximal medical management (e.g. beta blockers, ICD) - none alter disease progression



Note: Danon disease is a life-threatening and seriously debilitating condition. Above are not meant to be a strict categorization of symptoms/outcomes by age. Heart failure and death from Danon disease can occur well before the patient reaches his twenties. **Of note, the four adult patients in the Phase 1 study with 2.5-3.5 yr follow-up post gene therapy are currently alive, clinically stable, and free from Danon disease progression at ages 21-24 years old – in contrast to the trend in the natural history of this disease.**

NYHA = New York Heart Association Class; ICD: implantable cardioverter-defibrillator; HTx = Heart Transplantation; VAD: Ventricular Assist Device

Figure created based on data from Brambatti M et al. Int J Cardiol. 2019;286:92-98; Boucek D, Jirikowic J, Taylor M. Genet Med 2011; 13(6):563-68

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Phase 1 Data: Benefit Observed Across All Key Clinical Parameters

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

Cohort	Patient ID	Follow-up (months)	**Myocardial LAMP2 Grade (≤M12)**	TnI Δ (≤M12)	BNP Δ (≤M12)	**LV mass Δ (g)**	**LV Mass Index Δ (g/m^2.7)**	Max LV Wall Thickness Δ (mm)	NYHA class Δ	KCCQ score Δ
Low dose adult/ adolescent	1001	36	1	-75% (M18)	-36%	311 ⇢ 212	85 ⇢ 57	25 ⇢ 23	II ⇢ II	44 ⇢ 49
	1002	36	3	-79%	-76%	989 ⇢ 511	260 ⇢ 129	64 ⇢ 38	II ⇢ II	64 ⇢ 81
	1005	30	2 (M9)	-57% (M9)	-64% (M9)	438 ⇢ 375	98 ⇢ 76	33 ⇢ 24	II ⇢ I	77 ⇢ 85 (M24)
High dose adult/ adolescent	1006	24	1	-47%	-70%	410 ⇢ 300	90 ⇢ 63	22 ⇢ 18	II ⇢ I	79 ⇢ 82
Low dose pediatric	1008	12	1	-86%	-83%	605 ⇢ 447	140 ⇢ 96	42 ⇢ 39	II ⇢ I	50 ⇢ 82
	1009	6	1	-90%	-62%	234 ⇢ 185	83 ⇢ 63	20 ⇢ 20	II ⇢ I	52 ⇢ 78

All specified parameters either improved or stabilized (none deteriorated)

Improved Stabilized Worsened

Does not include pt 1007 in Ph1 trial who had advanced HF with EF<40% at enrollment and received HTx 5M following tx due to pre-existing advanced HF. Patient is currently stable. BNP, brain natriuretic peptide; hsTnI, high-sensitivity troponin I; KCCQ, Kansas City Cardiomyopathy Questionnaire; LAMP2, lysosome-associated membrane protein 2; LV, left ventricle; NYHA, New York Heart Association. Data cut-off Oct 6, 2022; Grade 0=negative staining; Grade 1 ≤25%; Grade 2 =26%-50%; Grade 3 =51%-75%; Grade 4 >75%. Low dose = $6.7x10^{13}$ GC/kg, high dose = $1.1x10^{14}$ GC/kg

Latest Pediatric Data Shows Sustained Improvements in Biomarkers, Symptoms, and Function (Updated Data)

Subject ID: A501-008-1008

Baseline Characteristics

AGE AT INFUSION	MAX LV WALL THICKNESS
12.3 years	41.9 mm, z-score +32
ICD yes[1]	6MWT
WPW yes	438 meters

18 months

Variable	Baseline[2]	Most Recent Follow-up
LAMP2 protein[3]	0	1 (M12)
Troponin-I (ng/mL)	1.89	0.30 (-84%)
BNP (pg/mL)	1837	328 (-82%)
NYHA Class	II	I
LV Mass Index	140	96 (-31%)[4]

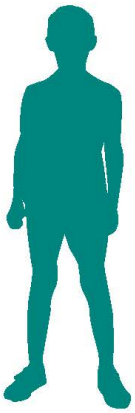
Subject ID: A501-008-1009

Baseline Characteristics

AGE AT INFUSION	MAX LV WALL THICKNESS
11.7 years	19.8 mm, z-score +12
ICD no	6MWT
WPW no	553 meters

12 months

Variable	Baseline[2]	Most Recent Follow-up
LAMP2 protein[3]	0	1 (M6)
Troponin-I (ng/mL)	0.67	0.08 (-88%)
BNP (pg/mL)	297	163 (-45%)
NYHA Class	II	I
LV Mass Index	83	60 (-28%)[4]



[1] Recommended prior to enrollment; ICD implanted 3 months after RP-A501 infusion. [2] Baseline values for troponin-I and BNP are the mean values from all pre-dose visits. [3] Extent of LAMP2 expression grading: Grade 0 = negative staining, Grade 1 < 25%, Grade 2 = 26-50%, Grade 3 = 51-75%, Grade 4 > 75%. [4] M12 Note: All data preliminary, not yet validated.
6MWT, 6-minute walk test; BNP, brain natriuretic peptide; ICD, implantable cardioverter defibrillator; KCCQ, Kansas City cardiomyopathy questionnaire; LAMP2, lysosome-associated membrane protein 2; LV, left ventricle; NYHA, New York Heart Association; WPW, Wolff-Parkinson-White syndrome.

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FDA Engagement and Alignment on Danon Program

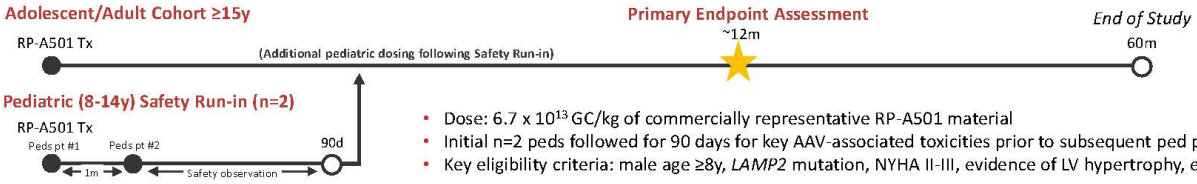
Since the EOP1 Meeting in November 2022, Rocket has had collaborative formal and informal discussions with FDA to align on the optimal pivotal Phase 2 study design

Previously Disclosed Trial Elements	Recent Engagement and Alignment
✓ *Pivotal, single-arm study*	✓ *F2F meeting with review team and senior FDA leadership*
✓ *Peds safety run-in*	✓ *Co-primary endpoint to support accelerated approval consisting of LAMP2 expression & LV Mass reduction of ≥ 10%*
✓ *Dosage ($6.7x10^{13}$ GC/kg)*	✓ *N=12 patients for pivotal study with potential for primary endpoint readout at 12 months*
✓ *Safety protocol & management plan*	✓ *Study to support AA with a path towards conversion to full approval (with longer follow-up)*
✓ *CMC (product comparability and potency assay)*	
✓ *NHS to serve as external comparator*	
✓ *RMAT designation granted*	

Phase 2 Trial Design – 12 Patients with 12-month Primary Endpoint Duration

Pivotal, global, single-arm, open label study with external comparator

PIVOTAL PHASE 2 STUDY DESIGN



- Dose: 6.7 x 10^{13} GC/kg of commercially representative RP-A501 material
- Initial n=2 peds followed for 90 days for key AAV-associated toxicities prior to subsequent ped pt enrollment
- Key eligibility criteria: male age ≥8y, *LAMP2* mutation, NYHA II-III, evidence of LV hypertrophy, elevated hsTnI

CO-PRIMARY ENDPOINT (AA)

- LAMP2 protein ≥ Grade 1 (IHC) AND
- Left Ventricular Mass (LV Mass): ≥10% ↓

SECONDARY & EXPLORATORY ENDPOINTS

- hs-troponin I (key secondary)
- Natriuretic peptides
- QoL instruments (KCCQ, PedsQL, PGI-C, PGI-S)
- NYHA Class
- 6MWT
- Event free survival
- Treatment emergent safety events
- Actigraphy

RISK MANAGEMENT PLAN, TRIAL OVERSIGHT

- Immunomodulatory regimen of Rituximab, Sirolimus, corticosteroids.
- Clinical monitoring team to closely monitor labs, clinical sequelae for AAV-associated toxicities.
- IDSMC: expertise in adult and pediatric cardiomyopathy, immunology, and biostatistics

CONCURRENT NATURAL HISTORY STUDY

Tx, treatment; LV, left ventricular; NYHA; IDSMC, Independent Data Safety and Monitoring Committee; hs-troponin I; KCCQ; HF; m, month; y, year; pts, patients

Prospective, Retrospective Natural History Study as External Comparator

Allows for robust comparisons and aligned with FDA guidance

Prospective Natural History Study

9 Males + 1 Female

Initiated in 2019, study complete

US Retrospective Natural History

51 Males + 67 Females

Initiated in 2006

EU Retrospective Natural History Study

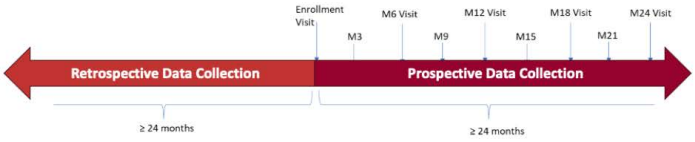
21 Males + 38 Females

Enrollment complete; analysis in Q4 2023

To be expanded through an additional prospective Rocket-initiated natural history study

Key Elements of Study Design:

- Entry criteria and endpoints similar to Phase 2 trial
- Appropriate matching to ensure robust comparisons
- Retrospective data collection to supplement prospective evaluation to ensure sufficient comparative data



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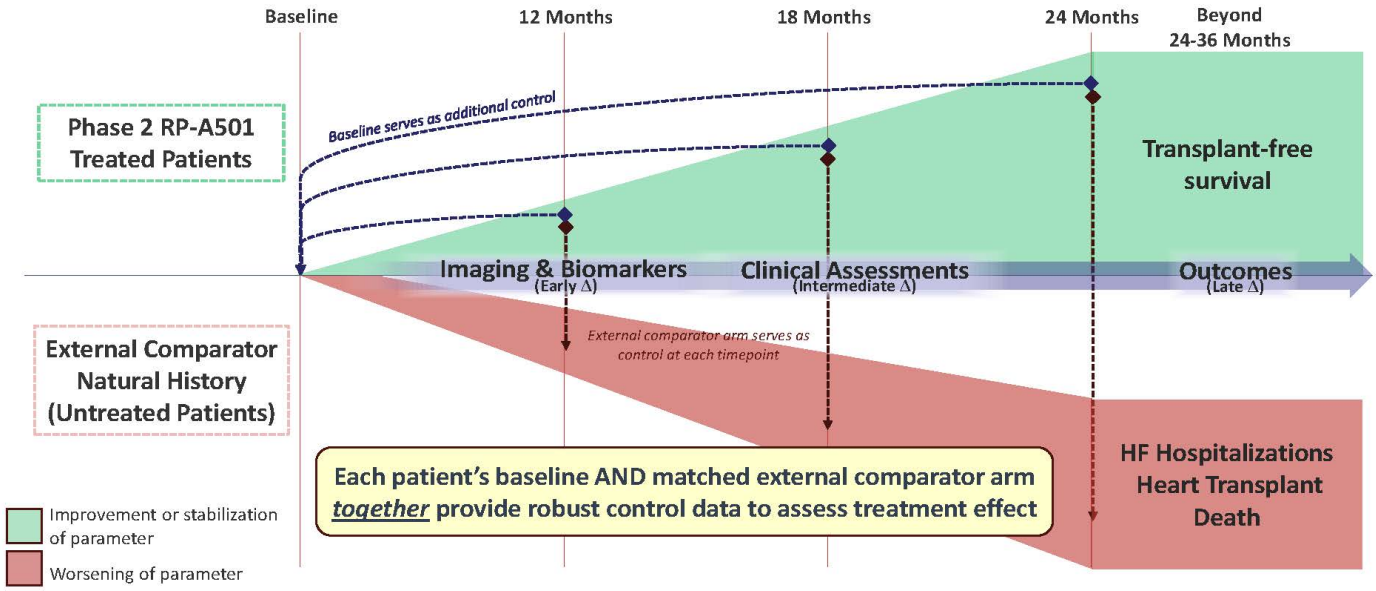
HTx, heart transplant; LVEF, LVEF, left ventricle ejection fraction.

Totality of Evidence to Demonstrate Treatment Effect



Note: Illustrative representation of potential treatment effects versus natural history

Co-Primary Endpoints for Accelerated Approval

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Primary Endpoint Is Reasonably Likely to Predict Clinical Benefit

Justification for use of LAMP2 protein expression and LV Mass

WT Full Length LAMP2 Protein Expression

- Mutation of *LAMP2* is root cause of Danon disease
- Epidemiologic support: even modest levels of LAMP2 confer a 2-decade survival advantage in female patients
- RP-A501 delivers full coding sequence of WT *LAMP2* gene
- Pre-clinical LAMP2 restoration conferred histologic, functional and survival benefits in LAMP2 knock-out model[1]
- Phase 1: LAMP2 expression associated with decreased vacuolar area, improved myofibrillar disarray, clinical improvement

Left Ventricular Mass

- Largest known hearts are Danon disease hearts
- Severity of the cardiomyopathy in Danon disease is the major prognostic factor[2]
- Retrospective natural history shows year-over-year increases in LV mass in Danon disease patients
- Phase 1: Consistent and significant reductions in LV mass as early as 6 months by echocardiography and cardiac MRI

Primary Endpoint Will Be Interpreted in a Clinical Context:

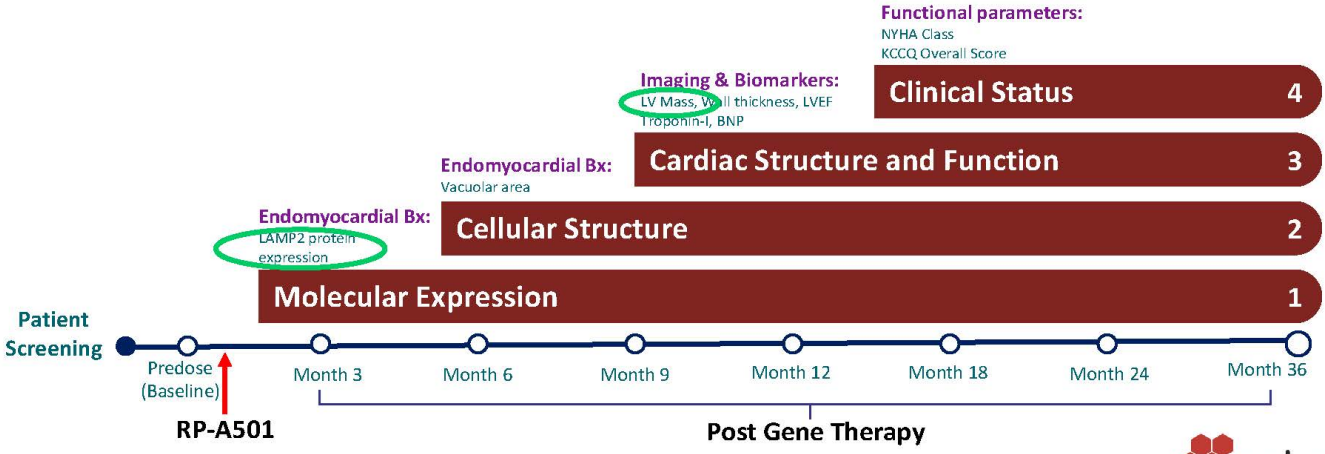
- *All components are measurable and unlikely to improve in the absence of a true treatment effect*
- *Primary endpoint will be assessed in the context of biomarkers, symptoms, QOL, clinical events derived from secondary endpoints and concurrent natural history study*
- *Phase 1 trial: LAMP2 expression and LV Mass improvements seen as early as 6 months in pediatric subjects with updated immunomodulation regimen*

[1] Manso 2020. *Sci Transl Med.*; [2] D'souza 2017. *J Community Hosp Intern Med Perspect.*

Mechanistic Pathway: Protein Expression to Cardiac Structure to Clinical Outcomes

Improvements Across Cellular, Cardiac Imaging and Functional Measures in Phase 1 Study

LAMP2 expression and LV Mass are mechanistically linked to functional measures and clinical outcomes; reasonably likely to predict clinical benefit



Note: BNP, brain natriuretic peptide; Bx, biopsy; KCCQ, Kansas City cardiomyopathy questionnaire; LAMP2, lysosome-associated membrane protein 2; LV, left ventricle; LVEF, LV ejection fraction; LVPWd, LV posterior wall end diastole; MLVWT, maximal LV wall thickness; NYHA, New York Heart Association

LAMP2 Protein Expression as Co-Primary Endpoint

RP-A501 encodes full-length, wild-type LAMP2B; cardiomyocytes are non-dividing cells

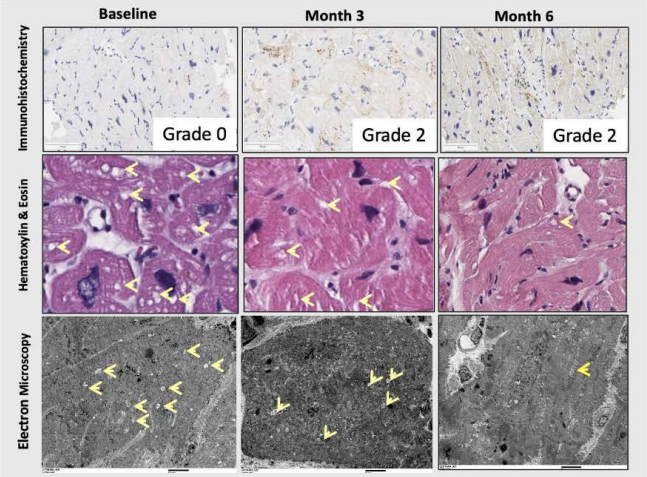
- ***LAMP2* mutation** and associated protein deficit is the **root cause** of Danon disease pathology
- **Grade ≥1 LAMP2** correlates with **evidence of efficacy** in Ph1
  - Absent expression at baseline (Grade 0) in all male patients
  - Improved cardiac biomarkers & hypertrophy, PRO/QOL and NYHA Class – sustained to 3+ years in adult patients, and 12+ months in pediatric patients
  - **Efficacy consistent** in patients with **Grade 1 vs. Grade >1 LAMP2** expression
- Ph1 expression correlates with ↓ autophagic vacuoles and improved myofibrillar disarray, cardiac biomarkers and hypertrophy
- In Danon females, **partial LAMP2 expression** associated with **~2 decade longer survival** than males

RP-A501 Increases LAMP2 Protein and Decreases Vacuolization

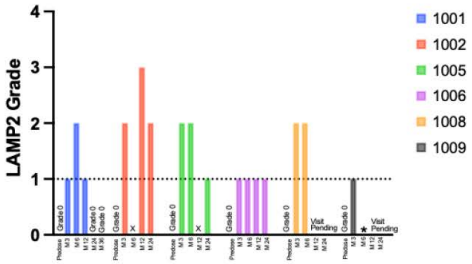
Enhanced autophagy leads to improved myocardial ultrastructure and clinical phenotype

Endomyocardial Biopsy Images

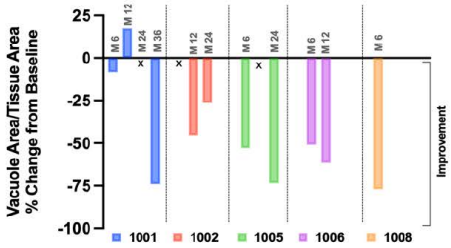
(Subject 1008, RP-A501 Phase 1 Study)



Myocardial LAMP2 Protein Expression



Vacuolar Area of Endomyocardial Tissue



Rocket AHA 2022 Poster Presentation reflects September 27, 2022 data cutoff. LAMP2, lysosome associated membrane protein 2; M, month. IHC = immunohistochemistry

Left Ventricle Mass as Co-Primary Endpoint

- Danon Disease is **fundamentally a disease of enlarged hearts**; the largest known hearts have been from Danon patients
- Left ventricular hypertrophy is the most consistent phenotypic **feature of disease progression**
- Left ventricular wall thickness has been shown to be a **significant predictor of CV events in cardiomyopathy**
- Meaningful **LV Mass decreases seen as early as 6-9M** in Phase 1 pediatric cohort, and LV Mass Index decreases **sustained to up to 36M in adult patients;** stark contrast to natural history
- In Phase 1 study, LV Mass **significantly correlated with improvements / stabilization in all parameters** including biomarkers (hsTnI, BNP), quality of life (KCCQ), and symptoms (NYHA)

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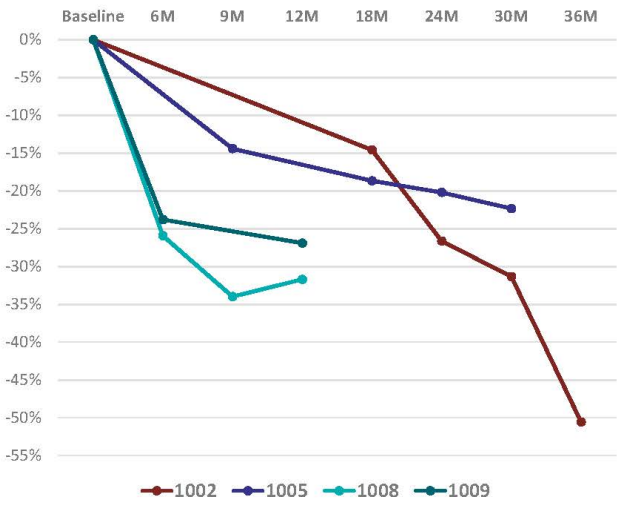
LVH Predicts Clinical Outcomes in HF and Cardiomyopathies

Cardiomyopathy or CVD	Measure of LVH	Event	HR; CI; p value
HFpEF (Shah 2019)	LV Mass index	CVD or HFH	HR 1.05 per 10 g/m(2); CI 1.00 to 1.10; p = 0.03
HFpEF (de Simone 2008)	LV Mass index	HF	HR 1.03; CI 1.02-1.04; p<0.00001
HCM (Liu 2016)	LV wall thickness	All Cause Death	HR 1.48; CI 1.01 to 2.17; p<0.05
HCM (Liu 2016)	LV wall thickness	CV Death	HR 2.17; CI 1.06 to 1.89; p<0.05
HCM (Liu 2016)	LV wall thickness	Sudden Cardiac Death	HR 3.17; CI 1.64 to 6.13; p<0.05
Fabry Disease (Orsborne 2022)	LV Mass index	Composite of CV events (HFH, MI, procedures, arrhythmias)	HR 1.008; CI 1.003-1.014; p=0.005
Fabry Disease (Hanneman 2020)	LV Mass index	Composite of CV events (HF, ventricular arrhythmia, cardiac death)	HR 1.1 per 5 g/m^2; CI 1.04-1.2; p<0.001

Shah A, JACC 2019; de Simone G. Eur Heart J. 2008; Liu, Q, Scientific Reports, 2016; Orsborne C, JACC, 2022; Hanneman K. Radiology. 2020

LV Mass Index in RP-A501 Phase 1 Study

RP-A501 Phase 1 Low-Dose Cohort: LV Mass Index % Change from Baseline[1]



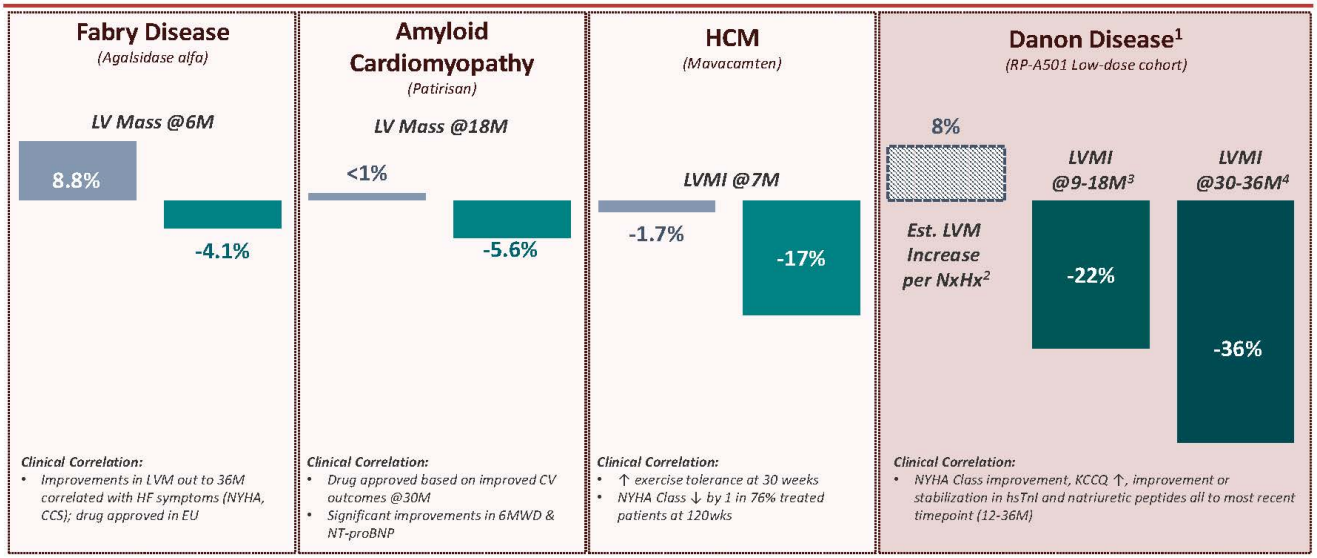
RP-A501 Phase 1 Study: LV Mass Index % CFB at ~12M
(9M or 18M where 12M not available)[2]

- >20% LVMI decrease observed as early as 6M in pediatric cohort, sustained to 12M timepoint
- Adult patients with appropriate immunomodulation show >10% LVMI decrease around 12M[2] with further decreases out to 30-36M of 22% to 51%

[1] Does not include patient 1001 (unmonitored immunomodulation). [2]12M visit data missing for 1002 and 1005 due to pandemic-related travel issues.

LV Mass / LV Mass Index (LVMI) Improves in DD with RP-A501

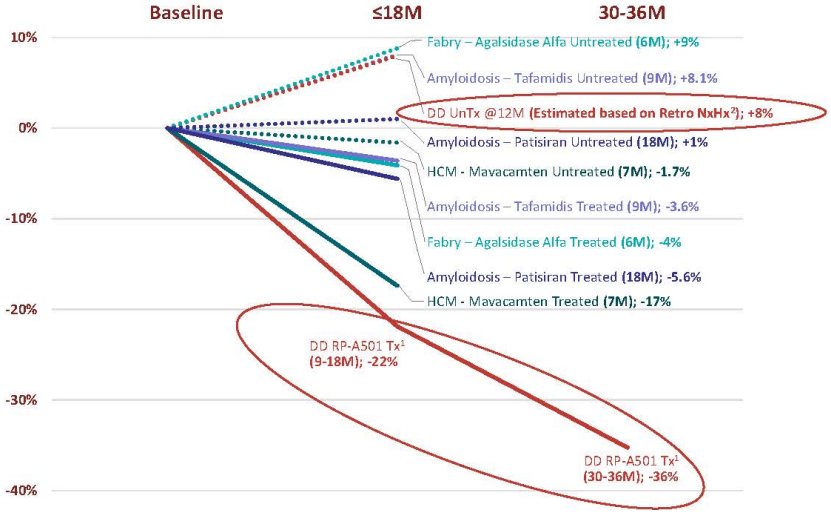
LV hypertrophy decreases greater than/comparable to other approved therapies



[1] RP-A501 Phase 1 low-dose cohort data; averages do not include 1001 (unmonitored immunomodulation) and 1006, 1007 (high dose patients). [2] Reflects estimated LV Mass increase over 12-18M for DD patients based on retrospective natural history data-set (shown on slide 20). [3] Reflects average of 1002 18M, 1005 9M, 1008 12M, 1009 12M. [4] Reflects average of 1005 30M, 1002 36M
Hughes 2008. Heart; Solomon 2019. Circulation; Saberi 2021. Circulation

LV Mass in RP-A501 Low-Dose Cohort Versus Recently Approved CV Therapies

LV Mass / LVMI Change from Baseline in Treated vs Untreated Patients: RP-A501 Low-Dose Cohort[1] and Recently Approved CV Therapies



- Data from Phase 1 study shows RP-A501 is potentially transformative for cardiac structure improvements and remodeling
- On par with recently approved therapies in other CV indications (across different disease etiologies and drug MOA's)

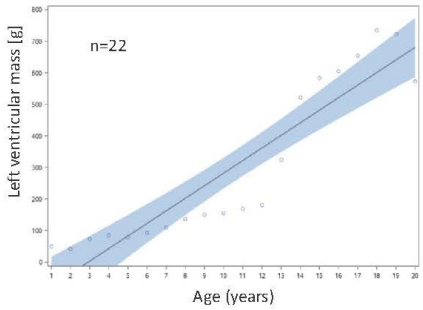
[1] *Averages do not include patient 1001 (unmonitored immunomodulation) and 1006, 1007 (high-dose cohort patients).*
[2] *Reflects estimated LV Mass increase over 12-18M for DD patients based on retrospective natural history data-set (shown on slide 20).*
Hughes 2008. Heart; Retti 2022. EHJ CV Imaging; Solomon 2019. Circulation; Saberi 2021. Circulation

Cardiac Imaging Captures the Progression of Danon Disease

Significant Increases in LV Mass and LV Wall Thickness Correlate with Age

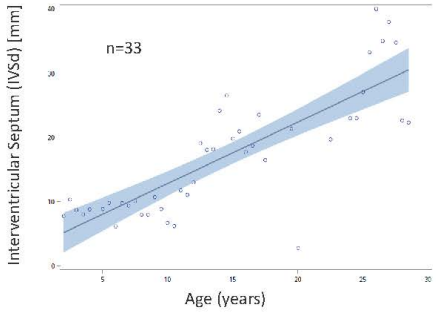
Trends in LV Mass

LV Mass increased by 39.86 ± 4.01 g per year



Trends in Septal Wall Thickness

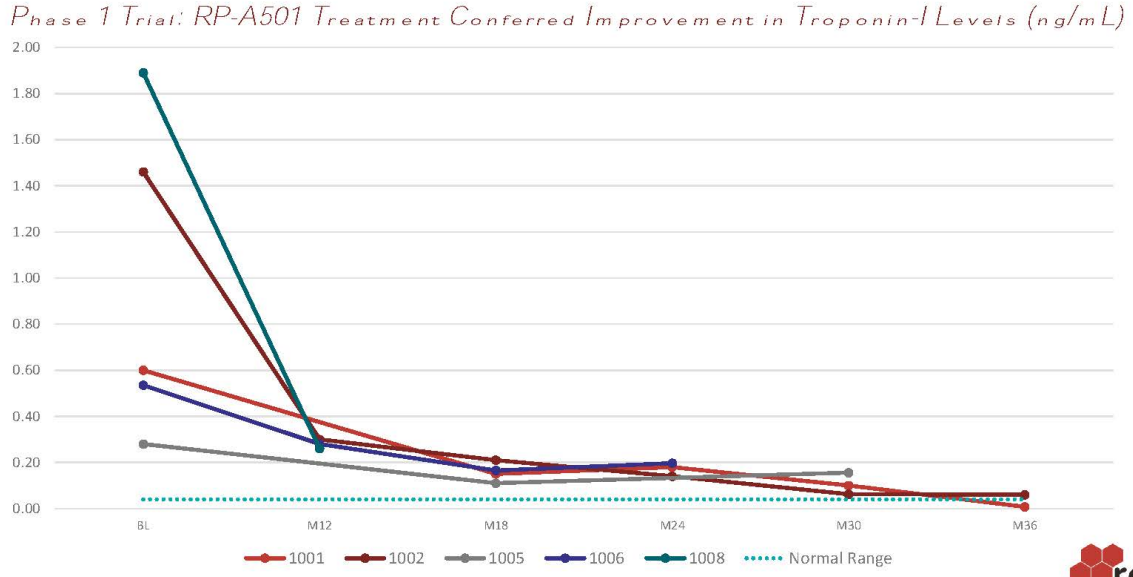
Septal wall increased by 0.96 ± 0.10 mm/year



Data from the Natural History Studies demonstrated increases in key echo parameters in DD that are known to predict disease progression in other types of cardiomyopathy

* Unpublished data from International Danon Disease Registry

Significant and Sustained Reduction in Troponin (Key Secondary Endpoint) Observed Across Patients in Phase 1 Study



Note: Upper limit of normal 0.04 ng/mL, BL = baseline

Current Status and Next Steps

rocket pharma

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Ongoing Danon Global Phase 2 Study Activities

Global Phase 2 Study (N=12)

United States

- ✓ RMAT, Fast Track, Orphan Drug Designations
- ✓ IRB approval at UCSD, CHOP; sites activated
- ✓ Multiple GMP lots manufactured in-house; sufficient drug product produced for full pivotal trial
- ✓ Global natural history study underway
  - ✓ FDA alignment on design
- ✓ Initiated pediatric safety run-in

Europe (UK + EU)

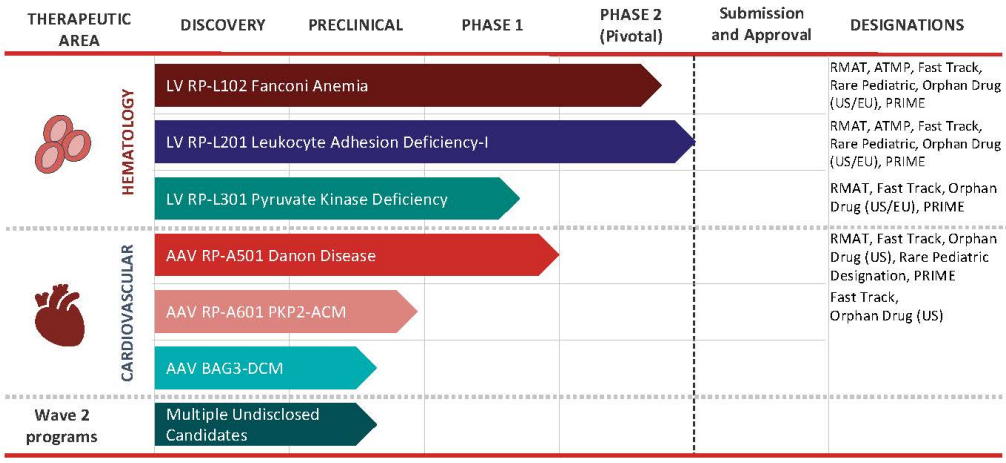
- ✓ Key regulatory designations obtained:
  - ✓ PRIME Designation
  - ✓ Orphan Drug Designation
  - ✓ Advanced Therapy Medicinal Product
- ✓ Scientific Advice Meeting held with EMA
  - ✓ On track for CTA/IMPD filing
- ✓ Multiple EU clinical site startup activities underway
- ✓ Successful QP audit of Rocket manufacturing facility; drug product cleared for EU use

Rocket Pipeline: 6 Disclosed Programs Across Two Platforms with Compelling Clinical and/or Pre-clinical Proof of Concept

Criteria used to select programs

- First-, best- and/or only-in-class
- On-target MOA; clear endpoints
- Sizeable market to maximize patient impact

6+ programs with 2 programs fast approaching commercialization



AAV, adeno-associated virus; ATMP, advanced therapy medicinal product; BLA, Biologics License Application; LV, lentiviral vector; MAA, Marketing Authorisation Application; MOA, mechanism of action; PRIME, PRIority MEdicines; RMAT, regenerative medicine advanced therapy. PKP2: plakophilin 2; ACM: Arrhythmogenic Cardiomyopathy; BAG3: BLC2-associated athanogene 3 DCM: Dilated Cardiomyopathy

Exhibit 99.2

Rocket Pharmaceuticals Reaches FDA Alignment on Pivotal Phase 2 Trial Design in RP-A501 in Danon Disease

Final alignment reached on a single arm, open-label study with natural history comparator and a biomarker based co-primary endpoint consisting of LAMP2 protein expression and Left Ventricular (LV) Mass

12 patient study with primary endpoint assessment at 12 months to support accelerated approval

CRANBURY, N.J. – **September 12, 2023** – Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a leading late-stage biotechnology company advancing an integrated and sustainable pipeline of genetic therapies for rare disorders with high unmet need, today announced that alignment has been reached with the Food and Drug Administration (FDA) on the global Phase 2 pivotal trial of RP-A501 for Danon Disease. Danon Disease is a uniformly fatal inherited cardiomyopathy that leads to mortality in the majority of male patients at age ~20 and females at age ~40, and for which there are no approved curative or disease-modifying therapies. The disease affects an estimated 15,000 to 30,000 patients in the U.S. and Europe.

"I am thrilled to announce our alignment with the FDA on the pivotal study design for RP-A501, which reflects the collaborative discussions with the review team and senior management at FDA's Center for Biologics Evaluation and Research and marks the first-ever regulatory pathway to approval for a genetic treatment for heart disease. We believe this milestone sets us on the most efficient and rapid path to delivering this potentially transformative therapy to Danon Disease patients who would otherwise progress to heart transplantation or death," said Gaurav Shah, M.D., Chief Executive Officer, Rocket Pharma. "I would also like to highlight the work conducted by our CMC team over the past several years to establish our in-house cGMP manufacturing capabilities, which has already provided us with sufficient material for the pivotal study and should support our eventual commercialization efforts."

Dr. Shah continued "As a one-time potentially curative infusion, RP-A501 has the potential to restore normal cardiac function and provide a lifetime of benefit to patients with Danon Disease who have no other viable treatment options. With today's progress in our Danon Disease program we believe we are forging a path to bring curative gene therapies to patients affected by devastating cardiovascular diseases and broadening the possibilities for addressing a larger array of inherited heart diseases through the promise of cardiac gene therapy."

Phase 2 Pivotal Trial of RP-A501 for Danon Disease

The global, single-arm, multi-center Phase 2 pivotal trial will evaluate the efficacy and safety of RP-A501 in 12 patients with Danon Disease, including a pediatric safety run-in (n=2), with a natural history comparator and a dose level of 6.7×10^{13} GC/kg.

- To support accelerated approval, the study will assess the efficacy of RP-A501 as measured by the biomarker-based co-primary endpoint consisting of improvements in LAMP2 protein expression (≥ Grade 1, as measured by immunohistochemistry), and reductions in left ventricular (LV) mass.
- Key secondary endpoints is change in troponin. Additional secondary endpoints will include natriuretic peptides, Kansas City Cardiomyopathy Questionnaire (KCCQ), New York Heart Association (NYHA) class, event free survival to 24 months and treatment emergent safety events. These endpoints could support a full approval with longer-term follow-up.
- A global natural history study will serve as an external comparator and run concurrently to the Phase 2 pivotal trial.
- In-house manufacturing has been completed with sufficient high-quality drug product produced to fully supply the Phase 2 pivotal study. Potency assays have been developed and qualified in accordance with FDA guidance.

Filing of the Clinical Trial Application (CTA)/Investigational Medicinal Product Dossier (IMPD) for RP-A501 to enable initiation of EU study activities is anticipated by the end of the third quarter of this year. Additionally, Rocket has secured an ICD-10 code from CMS for LAMP2 deficiency in Danon Disease.

About RP-A501
RP-A501 is Rocket's investigational gene therapy product for the treatment of Danon Disease and the first gene therapy for a cardiovascular condition to demonstrate safety and efficacy in clinical studies. Danon Disease is caused by mutations in the LAMP2 gene. RP-A501 consists of a recombinant adeno-associated serotype 9 (AAV9) capsid containing a full-length, wild-type version of the human LAMP2B transgene (AAV9.LAMP2B) which, when inserted into heart cells harboring mutations in the endogenous LAMP2B gene, has the potential to fully restore cardiac function at its root. RP-A501 represents a single dose treatment and is administered as an intravenous (IV) infusion. In preclinical and clinical studies, AAV9.LAMP2B has been shown to target cardiac cells (cardiomyocytes) and deliver the functional LAMP2B gene to heart tissue, which ultimately leads to improved cardiac structure and function in patients.

About Danon Disease
Danon Disease is a rare X-linked inherited disorder caused by mutations in the gene encoding lysosome-associated membrane protein 2 (LAMP-2), an important mediator of autophagy. This results in accumulation of autophagosomes and glycogen, particularly in cardiac muscle and other tissues, which ultimately leads to heart failure, and for male patients, frequent death during adolescence or early adulthood. It is estimated to have a prevalence of 15,000 to 30,000 patients in the U.S. and Europe. The only available treatment option for Danon Disease is cardiac transplantation, which is associated with substantial complications and is not considered curative. There is a high unmet medical need for patients with Danon Disease.

About Rocket Pharmaceuticals, Inc.
Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) is advancing an integrated and sustainable pipeline of investigational genetic therapies designed to correct the root cause of complex and rare childhood disorders. The Company's platform-agnostic approach enables it to design the best therapy for each indication, creating potentially transformative options for patients afflicted with rare genetic diseases. Rocket's clinical programs using lentiviral vector (LVV)-based gene therapy are for the treatment of Fanconi Anemia (FA), a difficult to treat genetic disease that leads to bone marrow failure and potentially cancer, Leukocyte Adhesion Deficiency-I (LAD-I), a severe pediatric genetic disorder that causes recurrent and life-threatening infections which are frequently fatal, and Pyruvate Kinase Deficiency (PKD), a rare, monogenic red blood cell disorder resulting in increased red cell destruction and mild to life-threatening anemia. Rocket's first clinical program using adeno-associated virus (AAV)-based gene therapy is for Danon Disease, a devastating, pediatric heart failure condition. Rocket also has received IND clearance for the AAV-based gene therapy program for PKP2-arrhythmogenic cardiomyopathy (ACM) and is advancing a preclinical program for BAG3-associated dilated cardiomyopathy (DCM). For more information about Rocket, please visit www.rocketpharma.com.

Rocket Cautionary Statement Regarding Forward-Looking Statements
Various statements in this release concerning Rocket's future expectations, plans and prospects, including without limitation, Rocket's expectations regarding the safety and effectiveness of product candidates that Rocket is developing to treat Danon Disease (DD), 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 Danon Disease program, including its planned pivotal trial, and the safety, effectiveness and timing of related pre-clinical studies and clinical trials, may constitute forward-looking statements for the purposes of the safe harbor provisions under the Private Securities Litigation Reform Act of 1995 and other federal securities laws and are subject to substantial risks, uncertainties and assumptions. You should not place reliance on these forward-looking statements, which often include words such as "believe," "expect," "anticipate," "intend," "plan," "will give," "estimate," "seek," "will," "may," "suggest" or similar terms, variations of such terms or the negative of those terms. Although Rocket believes that the expectations reflected in the forward-looking statements are reasonable, Rocket cannot guarantee such outcomes. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including, without limitation, Rocket's ability to monitor the impact of COVID-19 on its business operations and take steps to ensure the safety of patients, families and employees, the interest from patients and families for participation in each of Rocket's ongoing trials, patient enrollment, trial timelines and data readouts, our expectations regarding our drug supply for our ongoing and anticipated trials, actions of regulatory agencies, which may affect the initiation, timing and progress of pre-clinical studies and clinical trials of its product candidates, our ability to submit regulatory filings with the U.S. Food and Drug Administration (FDA) and to obtain and maintain FDA or other regulatory authority approval of our product candidates, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, our competitors' activities, including decisions as to the timing of competing product launches, pricing and discounting, our integration of an acquired business, which involves a number of risks, including the possibility that the integration process could result in the loss of key employees, the disruption of our ongoing business, or inconsistencies in standards, controls, procedures, or policies, our ability to successfully develop and commercialize any technology that we may in-license or products we may acquire and any unexpected expenditures, as well as those risks more fully discussed in the section entitled "Risk Factors" in Rocket's Annual Report on Form 10-K for the year ended December 31, 2022, filed February 28, 2023 with the SEC and subsequent filings with the SEC including our Quarterly Reports on Form 10-Q. Accordingly, you should not place undue reliance on these forward-looking statements. All such statements speak only as of the date made, and Rocket undertakes no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

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