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): January 9, 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 8.01. Other Events.

On January 9, 2023, Rocket Pharmaceuticals, Inc. (the "Company") updated information reflected in a slide presentation and a press release, which are attached as Exhibit 99.1 and Exhibit 99.2 to this Current Report on Form 8-K, respectively, and are incorporated herein by reference. Representatives of the Company intend to use the updated presentation and information contained in the press release in meetings with investors from time to time.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits.

99.1	Investor Presentation of Rocket Pharmaceuticals, Inc.
99.2	Press Release of Rocket Pharmaceuticals, Inc.
104	Cover Page Interactive Data File (embedded within the Inline XBRL document).

SIGNATURES

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

Rocket Pharmaceuticals, Inc.

Date: January 9, 2023

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

Exhibit 99.1


rocket
pharma
41st Annual J.P. Morgan Healthcare Conference Company Presentation
Gaurav Shah, MD
Chief Executive Officer
January 9, 2023

DISCLAIMER

Various statements in this presentation concerning Rocket's future expectations, plans and prospects, including without limitation, Rocket's expectations regarding its guidance for 2023 in light of COVID-19, the safety, effectiveness and timing of product candidates that Rocket may develop to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD), Danon Disease (DD), and other diseases, and the safety, effectiveness and timing of related pre-clinical studies and clinical trials and related data readouts, 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, our expectations regarding when clinical trial sites will resume normal business operations, our expectations regarding the delays and impact of COVID-19 on clinical sites, 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, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, and 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, 2021, filed February 28, 2022 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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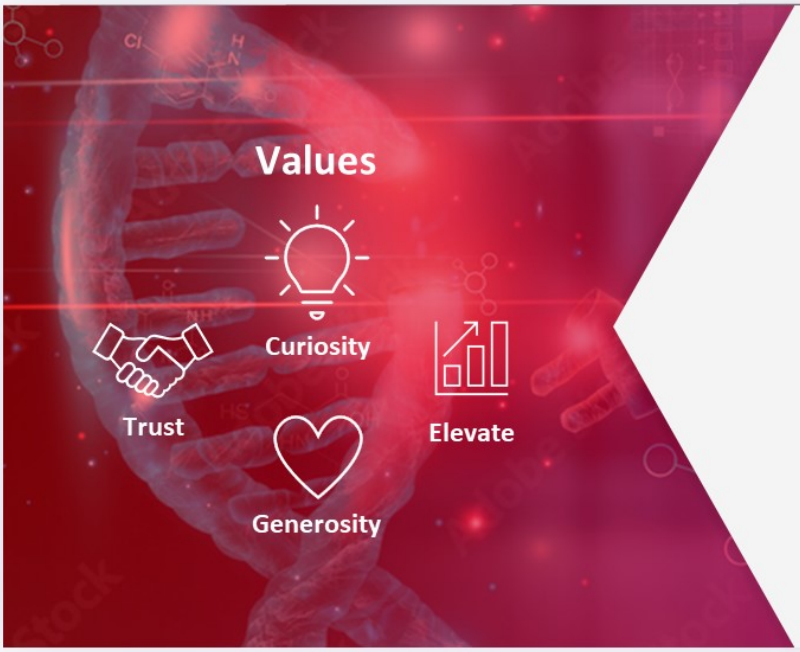
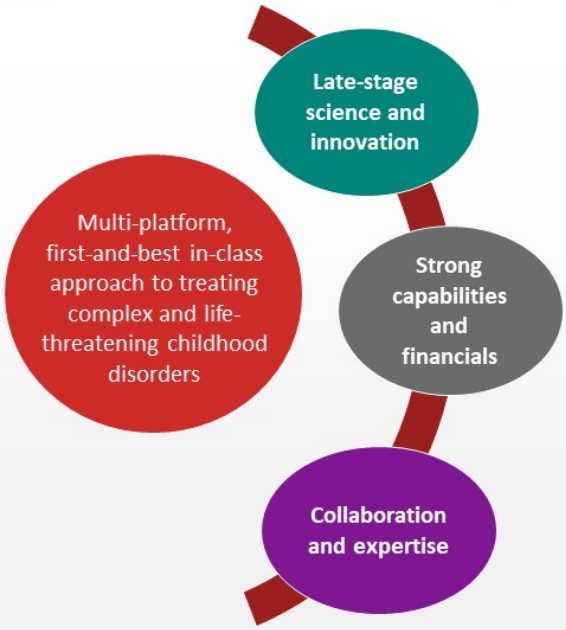
Vision: Seeking Gene Therapy Cures



Mission

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

Generating **Value-based** Gene Therapies



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

Therapeutic area focus: Heart and bone marrow
Only company with safety and efficacy data for gene therapy targeting the **heart**

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

Well capitalized to develop full pipeline of assets with
$401M[1]
in cash and cash equivalents; **sufficient to fund operations through 2024**

Leadership team with proven track record of
20+
drug approvals and launches

World-class scientific experts and partners learning from and collaborating with **patient communities**

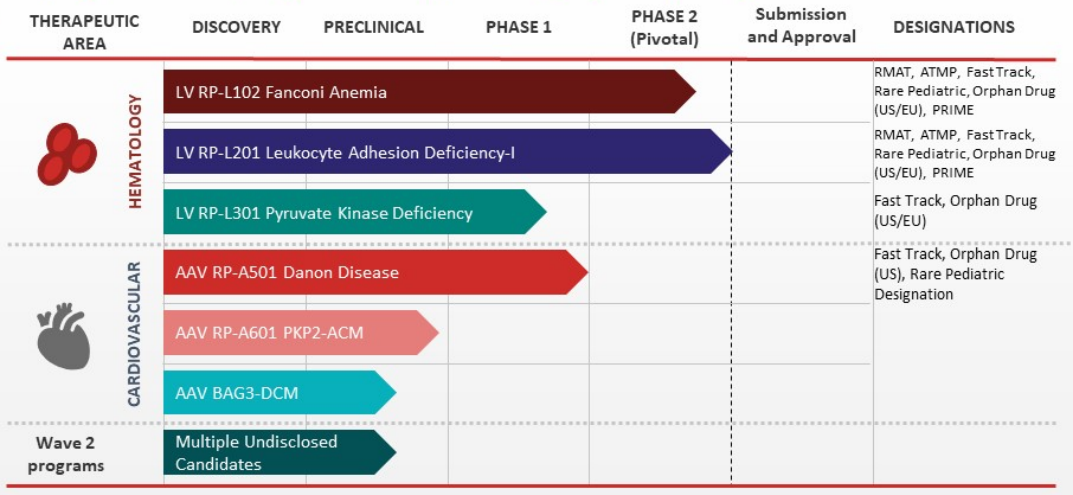
[1]Preliminary, unaudited cash balance as of December 31, 2022.

Strong Science, Carefully-selected Assets and Smart Execution:
6 Disclosed programs 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 regulatory filing and launch



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
Data on file. Rocket Pharmaceuticals. 2023.

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

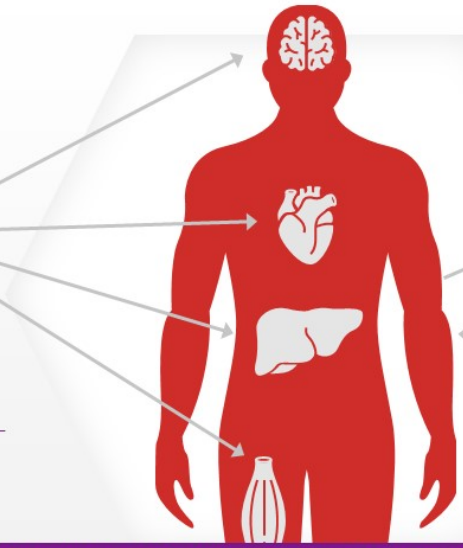
IN VIVO platform

Laboratory-produced AAV

Therapeutic AAV

Direct intravenous injection

RP-A501: Danon Disease
RP-A601: PKP2-ACM
AAV BAG3-DCM



EX VIVO platform

Remove cells and isolate patient HSCs

Laboratory-produced LV

Therapeutic LV

Gene-modified HSCs

Infusion of modified HSCs

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

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

AAV, adeno-associated virus; HSC, hematopoietic stem cell; LV, lentiviral vector.
Data on file. Rocket Pharmaceuticals. 2023

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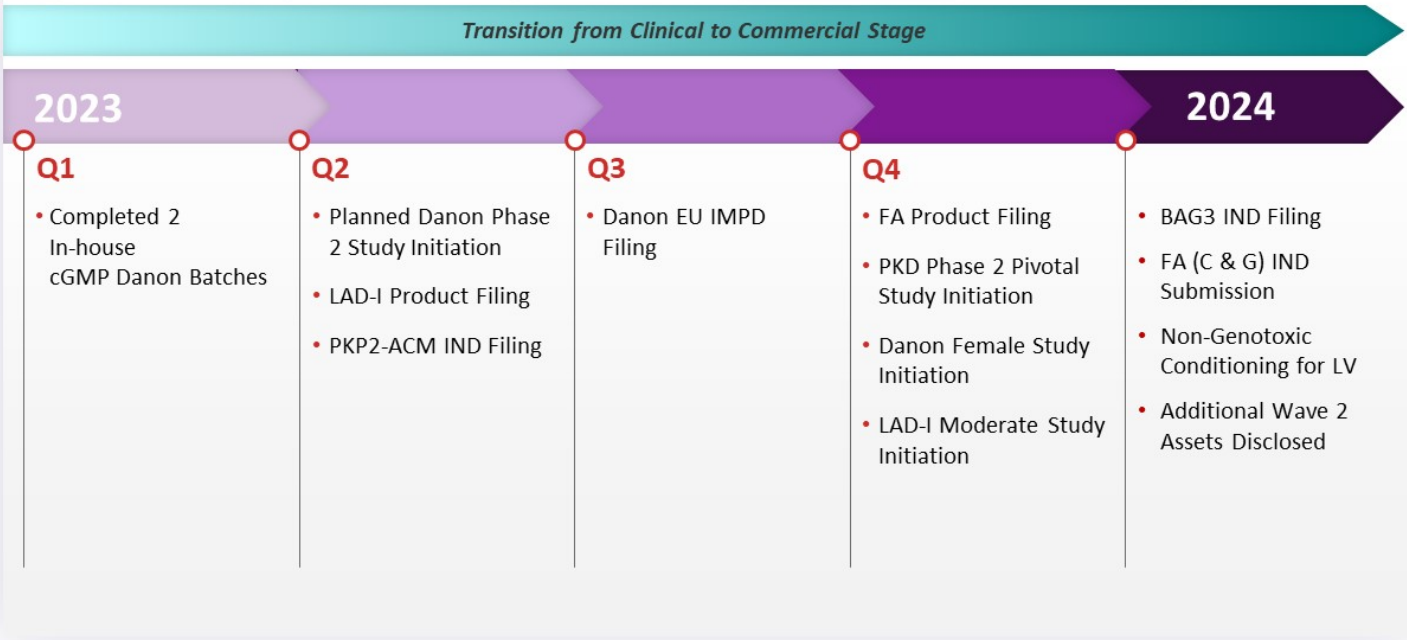
Looking Forward to a Catalyst-Rich 2023



cGMP, current Good Manufacturing Processes; FA, Fanconi anemia; H1, first half of the year; 2H, second half of the year; LAD-I, leukocyte adhesion deficiency-I; PKD, pyruvate kinase deficiency; Q1, first quarter of the yearQ2, second quarter of the year; Q3, third quarter of the year; Q4, fourth quarter of the year.
Data on file. Rocket Pharmaceuticals. 2023.

Danon Disease (DD): Serious Condition with Unmet Medical Need

Addressable Market – US and EU
Prevalence of **15,000 to 30,000** individuals
Annual incidence of **800 to 1,200** individuals

Disease Etiology

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

Therapeutic Challenges

- Standard of care:
  - Heart transplant (HTx)
- Limitations:
  - Considerable morbidity and mortality
  - Only ~20% of patients receive HTx
  - Not curative of extracardiac disease

Clinical Manifestations

Impaired autophagy

- Prominent autophagic vacuoles
- Myocardial disarray

Other clinical manifestations

- Skeletal myopathy
- CNS manifestations
- Ophthalmologic manifestations

Severe cardiomyopathy

- Mortality secondary to heart failure or arrhythmia
- Males: Aggressive disease course, median overall survival: 19 years
- Females: Delayed median presentation (~20 years later) due to additional X chromosome, highly morbid and fatal disorder

CNS, central nervous system; *LAMP-2B*, lysosome-associated membrane protein 2B.

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

Phase 1 Study: Treatment Completed

Non-randomized open label study in male DD patients

Pediatric
8 to 14 years
n=2 at CHOP

Adults (and Adolescents)
≥15 years
n=5 at UCSD

Single intravenous dose of RP-A501 (AAV9.LAMP2B) delivering full coding sequence of the *LAMP-2B* gene

****Enrollment Complete****

Cohort	Patient ID	Age at infusion	Time of follow-up (months)
Low dose ($6.7x10^{13}$ GC/kg) Pediatric	1008	12.3	12
	1009	11.7	6
Low dose ($6.7x10^{13}$ GC/kg) Adult and older adolescent	1001	17.4	36
	1002	20.3	36
	1005	18.3	30
High dose* ($1.1x10^{14}$ GC/kg) Adult and older adolescent	1006	21.1	24
	1007	20.7	N/A[†]

6 to 36 months

PRIMARY OUTCOMES

- **Early and long-term safety**
- Target tissue transduction and LAMP2B protein expression
- Improved myocardial histology
- Clinical improvement or stabilization

Data Reporting Details

- Pre-dose (baseline) value defined as the mean values from all visits prior to infusion
- Core lab data presented for echocardiographic parameters, cardiac serologies and cardiac histology

CHOP, Children's Hospital of Philadelphia; DD, Danon disease; *LAMP-2B*, lysosome-associated membrane protein 2B; UCSD, University of California San Diego.
*No further enrollment at this dose. †Due to advanced heart failure at the time of dosing (LVEF <40%), patient 1007 received a heart transplant 5 months following infusion of RP-A501. Patient is currently stable

RP-A501 Demonstrates Favorable Safety Profile With Enhanced Immunomodulation Protocol

ADULT COHORT

All SAEs observed within initial 2-4 months following dosing; reversible with supportive care

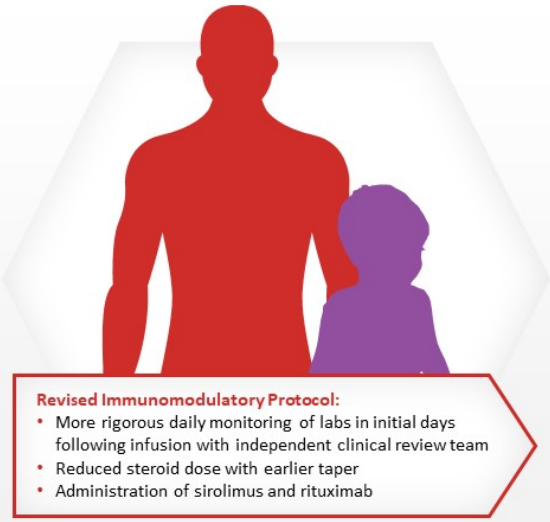
Low Dose

- **No SAEs related to drug product:** 2 steroid related SAEs (myopathy)

High Dose

- One instance of reversible TMA; led to enhanced RMP
- One instance of steroid myopathy

- **Both high and low-doses continue to be well tolerated at 2-3 years post treatment**
- **No additional SAEs observed following initial 2-4 months**



Revised Immunomodulatory Protocol:

- More rigorous daily monitoring of labs in initial days following infusion with independent clinical review team
- Reduced steroid dose with earlier taper
- Administration of sirolimus and rituximab

PEDIATRIC COHORT
(Low dose)

No RP-A501–related SAEs

All AEs were transient and reversible, with 8 and 13 months of follow up in 1008 and 1009, respectively

Platelets remained within normal range

No reported **skeletal myopathy or late transaminitis** with initial steroid dose reduction and more rapid taper, and introduction of sirolimus

- **Minimal complement activation**
- **No complement-related clinical or laboratory AEs**
- **All AEs were transient and reversible**
- **No treatment-related SAEs**

AE, adverse event; SAE, serious AE; TMA, thrombotic microangiopathy.
Data cut-off September 27, 2022, with source data verification through July 11, 2022.

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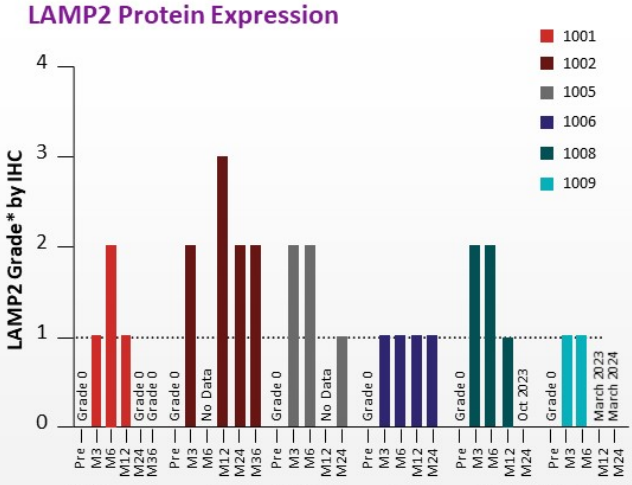
Pediatric LAMP2 Protein and DNA Suggests Durable Expression As Demonstrated in Adult Cohort

LAMP2 Protein Expression



*LAMP2 protein expression assessed (relative to normal human controls) by core lab in a blinded fashion of entire tissue sample; Percentages reflect estimated extent of LAMP2 staining: Grade 0=negative staining; Grade 1 ≤25%; Grade 2 =26%-50%; Grade 3 =51%-75%; Grade 4 >75%.

Cardiac LAMP2 DNA by qPCR

(vector copies per diploid nucleus)

Patient ID	Predose	Month 6	Month 12	Month 36
1001†	0	0.384	0.197	0.120
1002	0	ND	0.575	0.590§
1005	0	0.583	ND	1.228§
1006	0	2.693	1.131	-
1008	0	0.492	-	-
1009	0	Data pending	-	-

Note: Cardiomyocytes frequently multinucleated and/or have polyploid nuclei (several genome copies per cell); however, VCN is calculated assuming one diploid nucleus per cell. As a result, presented VCNs likely underestimated by factor of 2-4[1]; ND. not done, -, visit pending.

† Corticosteroid compliance uncertain. § Month 30 visit.

LAMP2, lysosome associated membrane protein 2; M, month. IHC= immunohistochemistry; qPCR = quantitative polymerase chain reaction; ND = not done.
The 2nd Cohort 2 pt (1007) underwent heart transplant due to DD progression five months post RP-A501 infusion; data post not included. Pt is currently clinically stable.
[1]Derks W, Bergmann O. Polyploidy in Cardiomyocytes: Roadblock to Heart Regeneration?. Circ Res. 2020;126(4):552-565.

Improvement or Stabilization Observed Across Key Biomarker, Echo Findings and Functional Measures in Phase 1 RP-A501 Study

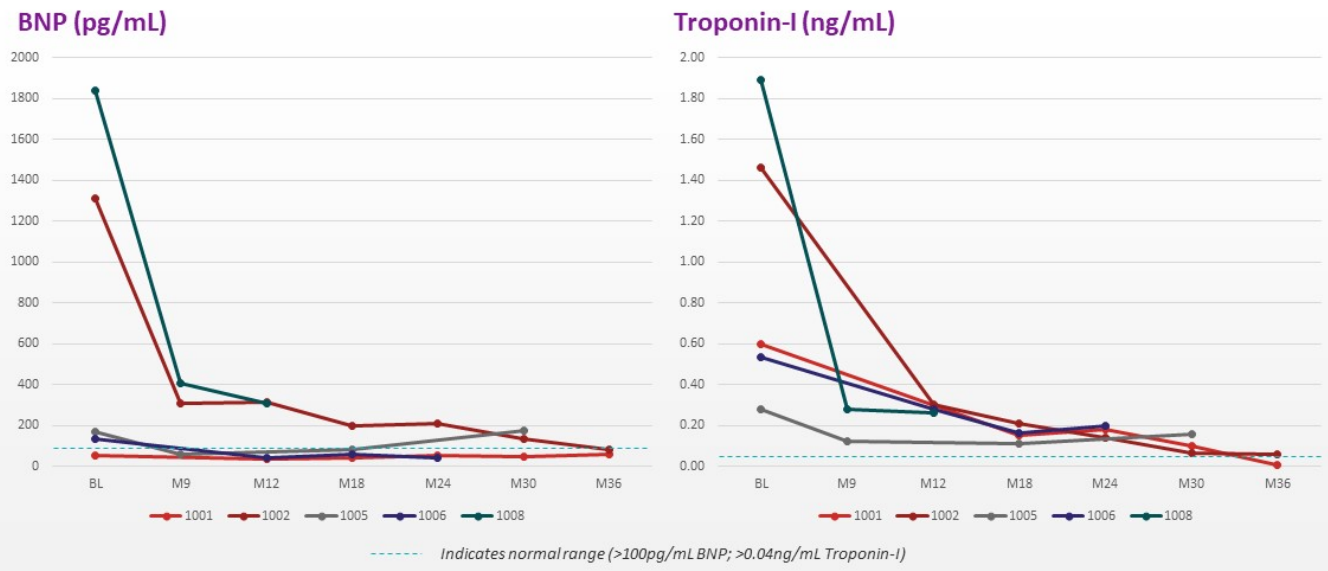
Cohort	Patient ID	Most recent visit (months)	Δ hsTnI	Δ BNP	Δ LV mass	Δ LV max wall thickness	Δ NYHA class	Δ KCCQ score
Low dose pediatric	1008	12	↓86%	↓83%	↓29%[1]	↓15%[1]	II ->I	+32.3
	1009	6	↓90%	↓62%	↓21%	↑3%	II ->I	+26
Low dose adult/ adolescent	1001	36	↓98%	↑8%	↓32%	↓9%	II ->II	+5.3
	1002	36	↓96%	↓94%	↓48%	↓40%	II ->I[2]	+17.8
	1005	30	↓46%	↑6%	↓14%	↓27%	II ->I	+8.3[3]
High dose adult/ adolescent	1006	24	↓63%	↓69%	↓27%	↓15%	II ->I	+3.1

Darker Green = improved; Lighter Green = minimal change (stabilization)

BNP, brain natriuretic peptide; DD, Danon disease; hsTnI, high-sensitivity troponin I; KCCQ, Kansas City Cardiomyopathy Questionnaire; LAMP2, lysosome-associated membrane protein 2; LV, left ventricle; NYHA, New York Heart Association. 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.

[1] 1008 echocardiographic parameters are M9 visit (M12 pending).
[2] 1002 NYHA class depicted for M30 visit (M36 pending).
[3] 1005 KCCQ score depicted for M24 visit (M30 pending).

Improvement or Stabilization Observed Across Key Cardiac Biomarkers



Note: Graphs do not depict serologic values during initial months after therapy; BL, baseline; M, month. 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.

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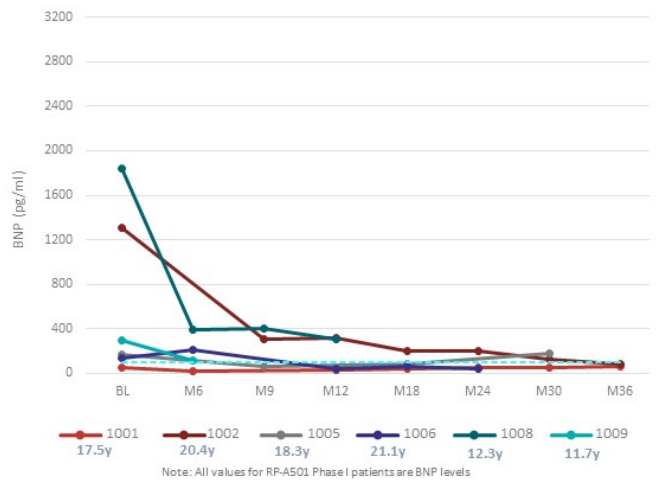
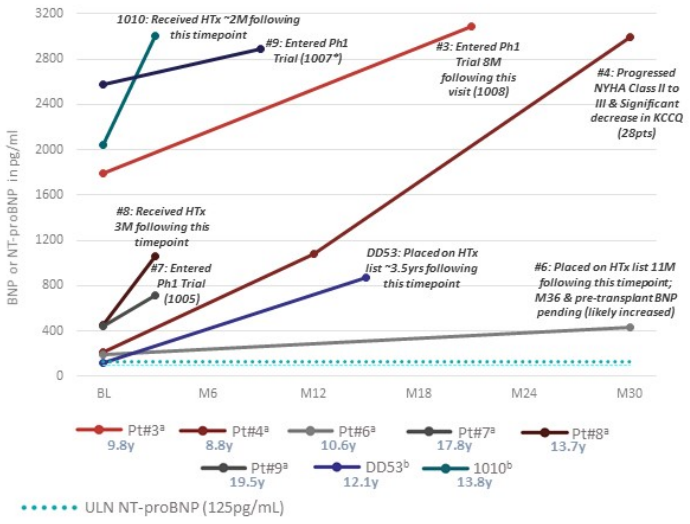
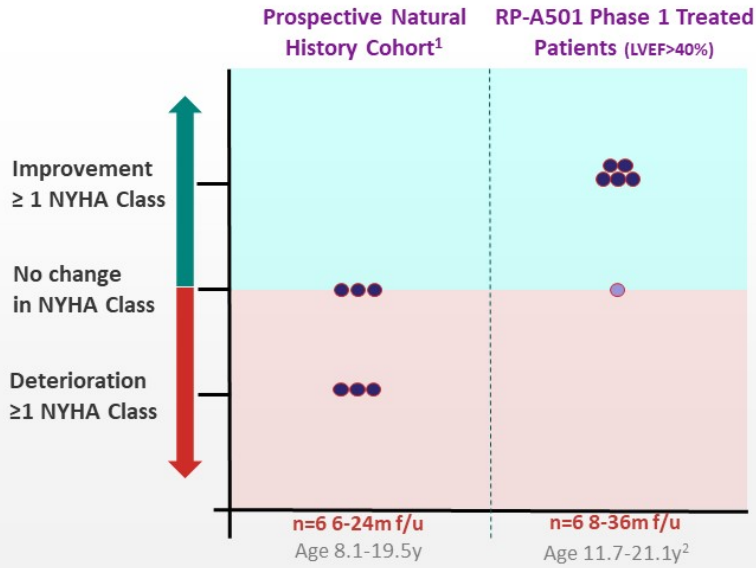
RP-A501 Phase 1 Patients: Marked Divergence from Natural History in Key Biomarkers





Note: [a]NT-proBNP tends to be higher than corresponding [b]BNP values given longer plasma half-life. External comparator data from prospective natural history (#s 3,4,6,7,8,9), natural history (DD53) and screen failure from Phase I study (1010). Phase I graph only reflects BNP levels and does not depict serologic values prior to initial 6 months after therapy. 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.

NYHA Class in Danon Disease Male Patients:
Natural History versus RP-A501 Phase 1



Prospective Natural History:
- **No patients had improved NYHA Class**

RP-A501:
- **All patients** with baseline LVEF ≥40% and monitored immunomodulation **had improved NYHA Class** (from Class II at baseline to Class I)

- Indicates pts with LVEF ≥40% at enrollment in prospective Nat Hx study or RP-A501 Phase 1
- Indicates pts with LVEF ≥40% at enrollment in RP-A501 Phase 1; unmonitored immunomodulation

Note: 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.
[1]Prospective natural history cohort: Sequential NYHA information was available for 6 of 9 patients in cohort. For remaining 3 patients, 1 was lost to follow-up, one received heart transplant (presumably deteriorated), and one was enrolled in the Phase 1 study.
[2]RP-A501: Pediatric patients age 11.7 and 12.3 years at rx; all other pts age 17.4-21.1 years at rx.

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Insights from Danon Disease Patients Treated on the Phase 1 Trial

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

Prior to therapy, he was afraid of dying and wanted a chance at life……After gene therapy, we see him smile more now, he bought his own place and working a couple of days a week, he has started to open up for meeting more friends in real life and has gotten a whole new peace of mind now …he feels better, and he didn't think that would ever happen **–Pt 1006**

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

He walked a 10K with his father following treatment. He is exercise training twice a week for an hour. **-Pt 1009**

Projected Pivotal Study Design

Key Agreements Reached with FDA

- ✓ $6.7x10^{13}$ GC/kg dose
- ✓ Single-arm, open-label study (randomization not appropriate)
- ✓ Support for use of natural history as external comparator information
- ✓ Potential for accelerated approval based on a composite biomarker-driven endpoint
- ✓ 6MWT, CPET are not appropriate endpoints in DD

Elements in Discussion

- Specific components of composite endpoint including LAMP2 expression
- Trial duration and time to endpoint
- 2 patient run-in for pediatric enrollment (age 8-14 years)

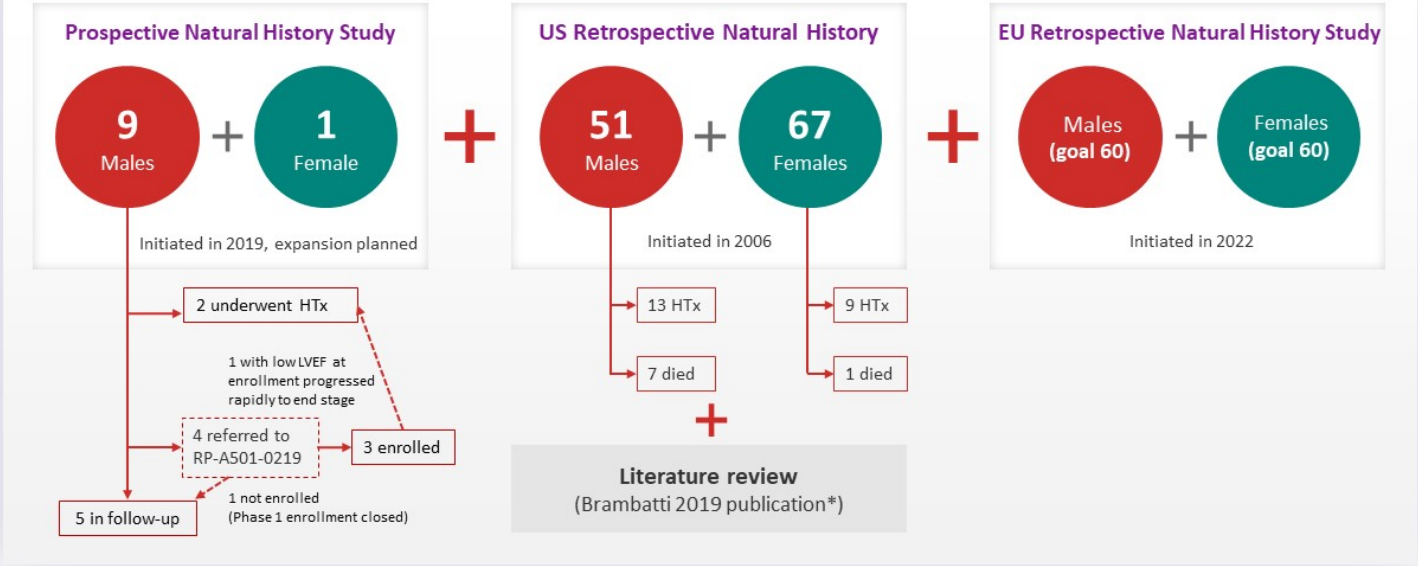
Confirmation pending submission of Phase II protocol and FDA review

Additional Study Elements

- Will utilize revised Phase I eligibility criteria (i.e. LVEF >50%)
- Age 8 years and older
- Optimized immunomodulatory regimen used in Phase I pediatric cohort
- All drug product will be produced in-house at Cranbury, NJ facility

Based on End of Phase I Regulatory Discussion and ongoing dialogue with FDA.

Robust Ongoing Natural History Efforts to Support External Comparator Sample



HTx, heart transplant; LVEF, LVEF, left ventricle ejection fraction.
*Brambatti M, Caspi O, Maolo A, et al. Danon disease: Gender differences in presentation and outcomes. Int J Cardiol. 2019;286:92-98.

In-House Manufacturing to Support Danon Pivotal Study and Commercial Production

- **2 Successful Danon AAV cGMP batches produced in Q4 2022**
- **Superior specifications to Phase I material**; allow for full dosing with lower total viral particles, potentially further improving safety profile
  - *Productivity:* ~3X increase in number of patient treatments per batch
  - *Product Quality:* Significant increase in full versus empty viral particles
  - *Product Comparability:* All attributes tested to date are comparable or improved
- **Regulatory progress and production capacity can support pivotal study and commercialization**
  - FDA clearance on continued utilization of HEK-293 cell-based process through commercial
  - FDA alignment on comparability approach
  - Potency assay developed in-house

Overall, in-house cGMP manufacturing delivers commercial-ready product with higher yield, improved quality, and likely enhances safety profile

Cranbury R&D and Manufacturing Facility Overview

- Total Lab Space: ~30,000 sq. ft. for process development, analytical development, MS&T and QC
- Manufacturing capability from small-scale to toxicology-scale material
- Streamlined tech transfer timeline for pipeline assets from plasmid selection to IND in <15 months
- Manufacturing expansion to add media and buffer production capability
- Incorporating fully automated in-house vial filler suite
- Anticipated 2X capacity increase in 2023

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

~100,000 ft^2

facility in Cranbury, NJ

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

Development Plan

Moving toward pivotal global Phase 2 study

Study Milestones

- ✓ Phase 1 treatment completed in males
- ✓ Orphan Drug, Rare Pediatric and Fast Track designations in the US (eligible for PRV)
- ✓ Completed 2 in-house cGMP batches
- ✓ End of Phase 1 Regulatory Meeting held with FDA

Ongoing Activities

- Final Phase 2 Study Design and Endpoints
- Initiate Phase 2 Global Pivotal Study Activities
- Expanded natural history study

GLOBAL REGISTRATIONAL PHASE 2 STUDY

FDA, Food and Drug Administration; PRV, priority review voucher.
Data on file. Rocket Pharmaceuticals. 2023.

RP-L102 for Fanconi Anemia Complementation Group A (FA-A)

Fanconi Anemia (A, C, and G)

Market Opportunity – US and EU
Prevalence of **5,500 to 7,000** individuals
Annual incidence of **200 to 275** individuals

Disease etiology

- **FA-A is an autosomal recessive disease caused by *FANCA* gene mutations**
- **FA proteins enable DNA repair**
- **FA-A accounts for 60% to 70% of FA cases**

Therapeutic challenges

Standard of care:
- Allogeneic HSCT

Limitations:
- Significant toxicities, especially for patients who do not have an HLA-identical sibling donor (~80%)
- 100-day mortality
- GvHD
- Increased long-term cancer risk

Clinical manifestations

Disorder of DNA repair characterized by:
- Progressive BMF; 80% of patients experience BMF within first decade of life
- Predisposition to hematologic malignancies and solid tumors

*Gene therapy approach: Selective advantage of corrected cells allows for **ex-vivo LV therapy without conditioning**; highly favorable benefit risk profile*

BMF, bone marrow failure; FA, Fanconi Anemia; FA-A, FA, group A; FANC, FA complementation group; GvHD, graft-versus-host disease; HLA, human leukocyte antigen; HSCT, hematopoietic stem cell transplantation.
Alter BP, Giri N, Savage SA, et al. Malignancies and survival patterns in the National Cancer Institute inherited bone marrow failure syndromes cohort study. Br J Haematol. 2010;150(2):179-188.

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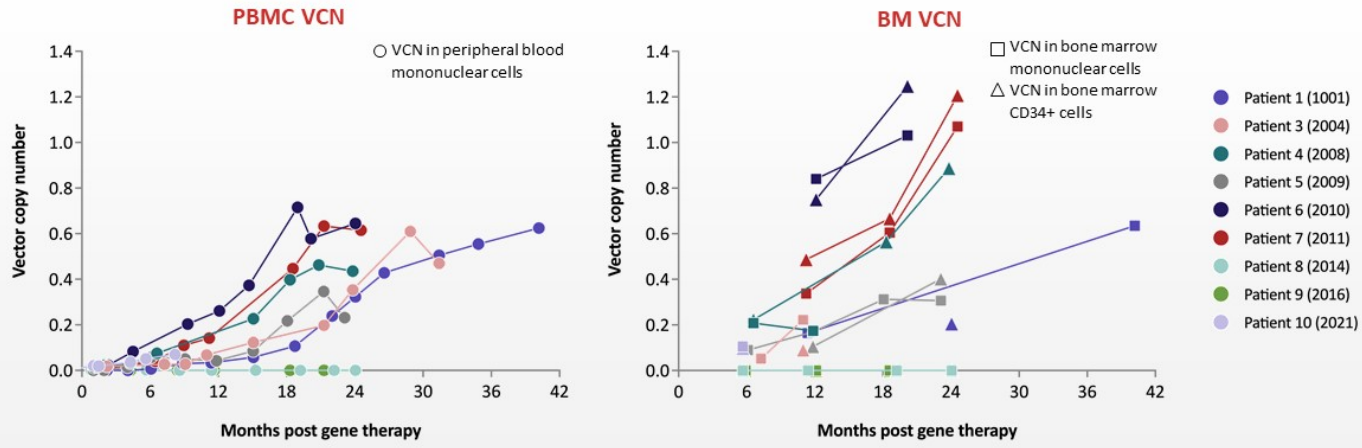
Progressively Increasing and Sustained Genetic Correction in 7 of 10 Patients ≥1 Year Post–RP-L102

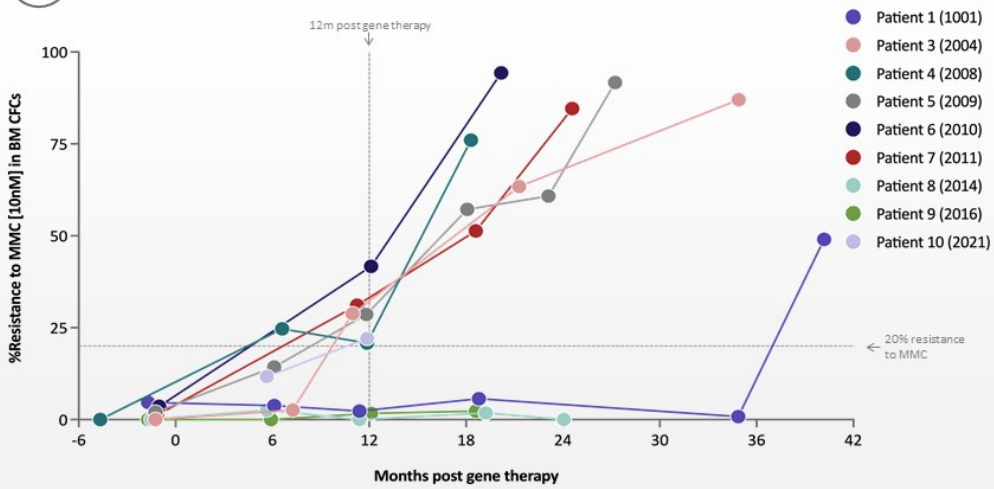
Progressive increases in gene markings in PB and BM in 7 patients



BM, bone marrow; CD34, cluster of differentiation 34; PB, peripheral blood; PBMC, PB mononuclear cells; VCN, vector copy number.
Not shown: PB and BM VCN in Patient 2 (1002), who was withdrawn from the study at 18 months post–RP-L102 infusion.
BM VCN not available at some stipulated time points due to insufficient sample to run assay.

Increasing Phenotypic Correction (MMC-resistance) over 1 to 3 Years Post–RP-L102

Sustained BM CFC MMC resistance observed in at least 6 patients*



For 5 patients, increased BM CFC MMC resistance ranging from 51% to 94% was observed at 18 to 24 months post–RP-L102 administration

MMC resistance of >20% achieved at 2 consecutive timepoints ≥12 months for n=5

BM, bone marrow; CFC, colony-forming cells; MMC, mitomycin-C.
Not shown: MMC resistance in Patient 2 (1002), who was withdrawn from the study at 18 months post–RP-L102 infusion.
*One additional patient (Patient 1: 1001) was noted to have BM MMC resistance of 49% at ~40 months post–RP-L102 infusion.

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Development Plan

Moving toward BLA/MAA filing

INITIAL EFFICACY AND HIGHLY FAVORABLE SAFETY PROFILE

- Initial comprehensive efficacy in 6/10 evaluable patients (≥12-month follow-up)
- No cytotoxic conditioning, only 1 transient RP-L102 related SAE (Grade 2)

TOP-LINE DATA READOUT ACHIEVED

- Rejection of null hypothesis with minimum of 5 patients with increased MMC resistance >10% at 2 timepoints between 12 and 36 months

NEXT STEPS

- **Update: CMC and clinical FDA discussions support BLA activities**
- 2 patients to be treated with product from commercial cell processing site in preparation for US launch

Anticipated simultaneous BLA/MAA filings

Additional life-cycle management activities:

- Expansion to FANC C and G
- Exploration of non-genotoxic conditioning and HSC expansion

REGULATORY DESIGNATIONS:

- RMAT, PRIME
- Orphan Drug designation in the US/EU
- Rare Pediatric Disease designation (eligible for PRV)
- Fast Track (US), ATMP

ATMP, advanced therapy medicinal product; BLA, Biologics License Application; CMC, chemistry, manufacturing, and controls; FANC, Fanconi Anemia complementation group; FDA, Food and Drug Administration; HSC, hematopoietic stem cell; MAA, Market Authorization Application; MMC, mitomycin-C; PRIME, PRIority MEdicines; PRV, priority review voucher; RMAT, Regenerative Medicine Advanced Therapy; SAE, severe adverse event.
Data on file. Rocket Pharmaceuticals. 2023.

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RP-L201 for LAD-I: *ITGB2* Gene Mutation

Market Opportunity – US and EU
Prevalence of **800 to 1,000** individuals
Annual incidence of **50 to 75** individuals

Disease etiology

- ***ITGB2* gene mutations (21q22.3), encoding the beta-2-integrin, CD18; essential for leukocyte adhesion to endothelium**
- **CD18 absent or reduced on neutrophils**

Therapeutic challenges

Standard of care:
- Allogeneic HSCT

Limitations:
- Donor availability
- Infections
- Frequent GvHD
- Graft failure

Clinical manifestations

Patients suffer from recurrent infections; fatal in majority
- Severe LAD-I: Death prior to age 2 in 60% to 75% of patients, infrequent survival >5 years in absence of allogeneic HSCT
- Moderate LAD-I: Death prior to age 40 in >50% of patients, extensive morbidity with recurrent infections and inflammatory lesions

CD18, cluster of differentiation 18; GvHD, graft-versus-host disease; HSCT, hematopoietic stem cell transplant; *ITGB2*, integrin subunit beta 2; LAD-I, leukocyte adhesion deficiency-I.

Almarza NE, Kasbekar S, Thrasher AJ, et al. Leukocyte adhesion deficiency-I: A comprehensive review of all published cases. J Allergy Clin Immunol Pract. 2018;6(4):1418-1420.e10.

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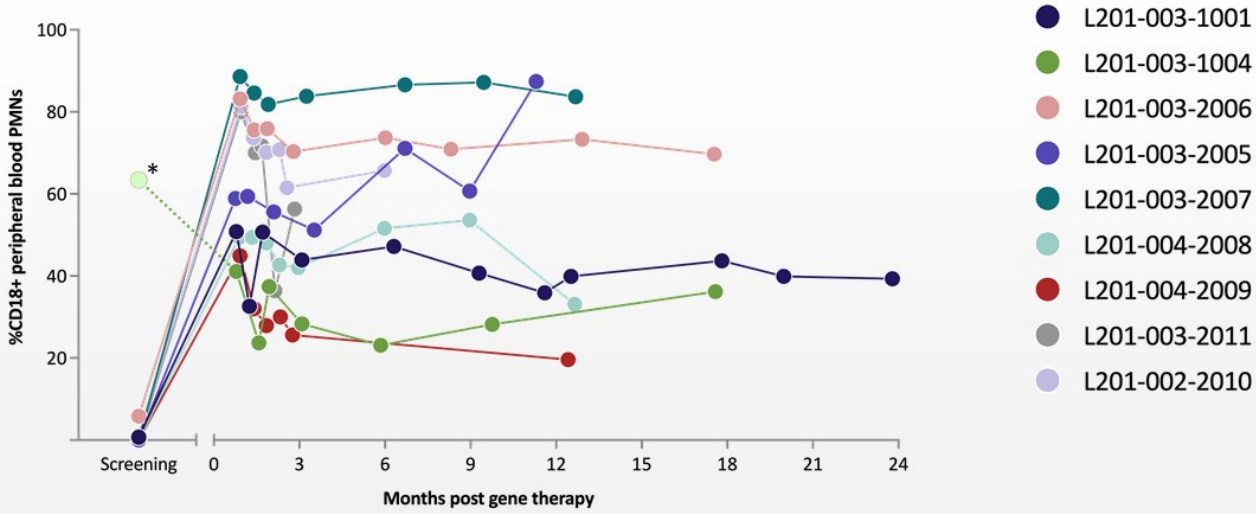
CD18 Expression in PB Polymorphonuclear Cells (PMNs)

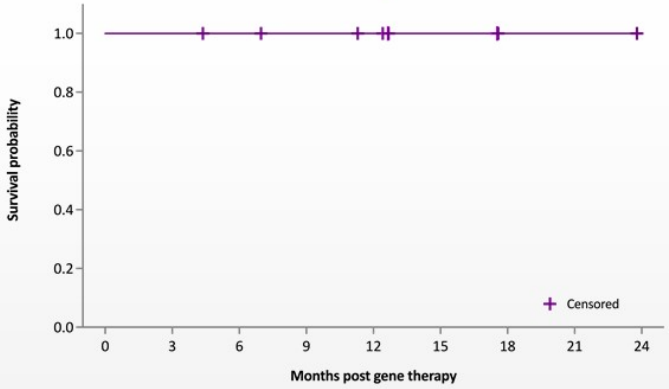
At 3 to 24 months after infusion, 9/9 patients sustained stable CD18 expression (median: 56%) with no therapy-related serious adverse events



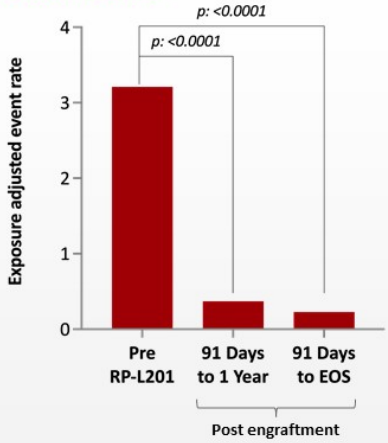
*Dim/weak CD18 expression reported at baseline for Subject L201-003-1004 in ~63% of cells in conjunction with <2% CD11a/CD11b expression, likely indicating abnormal/unstable protein.
LAD-I, Leukocyte Adhesion Deficiency-I; PB, peripheral blood; PMN, polymorphonuclear neutrophil.
Data on file. Rocket Pharmaceuticals. 2022. Data Cut-Off: April 6, 2022; Preliminary interim results are presented from the ongoing clinical study.

Significant Reduction in Hospitalizations and 100% Overall Survival

100% overall survival Kaplan–Meier estimate



Significant reduction in incidence of hospitalizations



Survival without allogeneic HSCT

Primary outcomes

- ≥2 years of age AND
- ≥1-year post–RP-L201 infusion

All patients have been able to stop prophylactic antibiotics

EOS, end of study; HSCT, hematopoietic stem cell transplantation; LAD-I, leukocyte adhesion deficiency-I.
Data cut-off: March 9, 2022.
Data on file. Rocket Pharmaceuticals. 2022.

Development Plan

Moving toward product filing

ENROLLMENT AND INITIAL EFFICACY

- Enrollment completed; 9/9 patients treated
- Efficacy observed in 9/9 patients with 3 to 24 months follow-up
- Efficacy is comprehensive, across all efficacy parameters including CD18 expression and survival

TOP-LINE DATA READOUT Q2 2022

- Survival for 9/9 patients, ≥2 years age and ≥1 year post-treatment
- No graft failure, GvHD
- No RP-L201 related SAEs

NEXT STEPS

- Progression to regulatory filing activities

Guiding Q2 2023 regulatory filing

Life-cycle management

- Potential label expansion to include moderate LAD-I population
- Potential study initiation in 2023

REGULATORY DESIGNATIONS:

- RMAT, PRIME
- Orphan Drug designation in the US/EU
- Rare Pediatric Disease designation (eligible for PRV)
- Fast Track (US), ATMP

ATMP, advanced therapy medicinal product; BLA, Biologics License Application; GvHD, graft-versus-host disease; LAD-I, leukocyte adhesion deficiency-I; MAA, Market Authorization Application; PRIME, PRIority MEdicines; PRV, priority review voucher; RMAT, Regenerative Medicine Advanced Therapy; SAE, severe adverse event.
Data cut-off: March 9, 2022.
Data on file. Rocket Pharmaceuticals. 2022.

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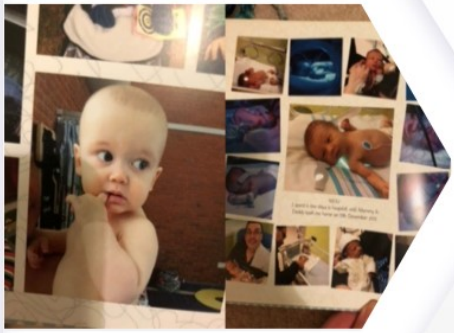
RP-L301 for PKD: ***PKLR*** Gene Mutation



Market Opportunity – US and EU
Prevalence of **4,000 to 8,000** individuals
Annual incidence of **75 to 125** individuals

Disease etiology

- Autosomal recessive inheritance
- Pyruvate kinase deficient RBCs cannot synthesize ATP, resulting in hemolytic anemia

Therapeutic challenges

- Standard of care: Chronic blood transfusions and splenectomy
- Limitations:
  - Iron overload
  - Extensive end-organ damage
  - Splenectomy confers lifelong infection and thrombotic risk

Clinical manifestations

- Lifelong chronic hemolysis
- Other clinical manifestations:
  - Anemia
  - Jaundice
  - Iron overload

ATP, adenosine triphosphate; PKD, pyruvate kinase deficiency; PKLR, pyruvate kinase L/R; RBC, red blood cell.
Zanella A, Fermo E, Bianchi P, Valentini G. Red cell pyruvate kinase deficiency: molecular and clinical aspects. Br J Haematol. 2005;130(1):11-25.

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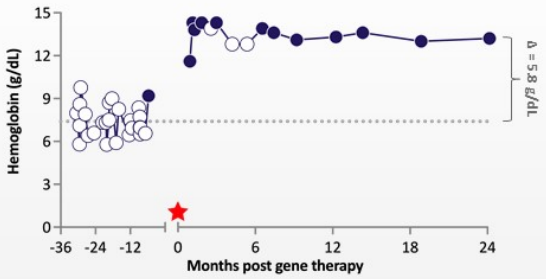
Preliminary Efficacy Results for Patients L301-006-1001 and L301-001-1002

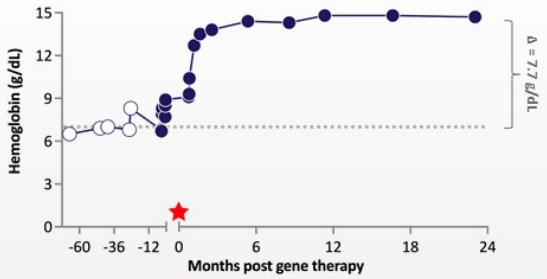
Hemoglobin improvement to normal range (from baselines in severe (<8 g/dL range)
Transfusion independence (extensive transfusion requirements prior to RP-L301)
Sustained improvement of hemolysis markers (LDH, bilirubin) and PB VCNs in 1.0 to 3.0 range

PATIENT 1001



- Sustained **hemoglobin normalization from ~7.4 g/dL to 13.2 g/dL** 24 months post–RP-L301 infusion
- No red blood cell transfusions required following engraftment

PATIENT 1002



- Sustained **hemoglobin normalization from ~7.0 to 14.7 g/dL** 24 months post–RP-L301 infusion
- No red blood cell transfusions required following engraftment

★ RP-L102 Infusion
.... Dotted lines indicate average hemoglobin for each patient prior to gene therapy

LDH, lactate dehydrogenase; PB, peripheral blood; VCN, vector copy number.
Data cut-off: October 26, 2022; preliminary interim results are presented from the ongoing clinical study.
Data on file. Rocket Pharmaceuticals. 2022.

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Development Plan

Moving toward pivotal Phase 2 study

PLAN FOR PHASE 2 AND LAUNCH

Key endpoints selected

- Hemoglobin increase
- ↓ 50% in transfusions or transfusion independence

Well-delineated natural history in recent PKD NHS publications

- Complete Phase 1 pediatric cohort dosing (N=2 to 3)
- End of Phase 1 regulatory meeting with FDA in 2023
- Approve and launch RP-L301; seek regulatory approval in the US and EU

REGULATORY DESIGNATIONS

Fast Track, Orphan Drug (US/EU), Rare Pediatric Disease (eligible for PRV)

LIFE-CYCLE MANAGEMENT

- Anticipated expansion study to pre-splenectomy patients
- Exploration of non-genotoxic conditioning

FDA, Food and Drug Administration; NHS, National Health Service; PKD, pyruvate kinase deficiency; PRV, priority review voucher.
Data on file. Rocket Pharmaceuticals. 2022.

Future Therapies: Wave 2 (AAV)

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
- 3 therapeutic areas (CV, heme and undisclosed)

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.
Data on file. Rocket Pharmaceuticals. 2022.

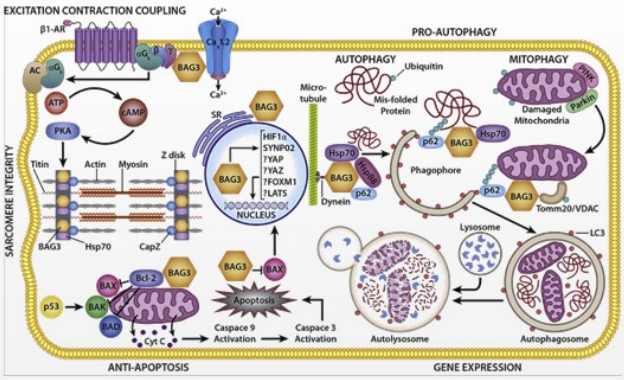
BAG3 Regulates Critical Functions in Cardiomyocytes

Cardiac contractility (1)

Enhances contractility by linking the β-adrenergic receptor and L-type Ca^{2+} channel

Structural support (2)

Provides support for the sarcomere by linking actin myofibrils with the Z-disc



Protein quality control (3)

Facilitates autophagy as a co-chaperone with heat shock proteins, recycling misfolded proteins

Anti-apoptosis (4)

Inhibits apoptosis (programmed cell death) through binding of BCL2

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

BAG3, BLC2-associated athanogene 3; BCL2, B-cell lymphoma 2.

Knezevic T, Myers VD, Su F, et al. Adeno-associated Virus Serotype 9 - Driven Expression of BAG3 Improves Left Ventricular Function in Murine Hearts with Left Ventricular Dysfunction Secondary to a Myocardial Infarction. JACC Basic Transl Sci. 2016;1(7):647-656.
Myers VD, Gerhard GS, McNamara DM, et al. Association of Variants in BAG3 With Cardiomyopathy Outcomes in African American Individuals JAMA Cardiol. 2018;3(10):929-938.

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BAG3-DCM Opportunity and Next Steps

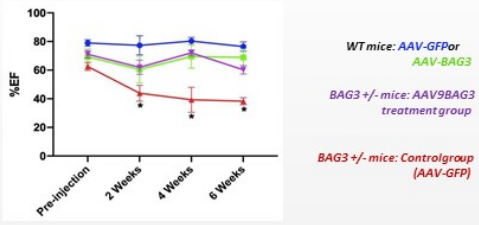
BAG3-DCM Represents a Significant Market with Unmet Need

- Dilated cardiomyopathy (DCM) is the most common form of cardiomyopathy
- 20% to 50% of DCM patients have familial DCM; up to 40% of whom have an identifiable genetic cause[1]
- Scientific societies recently endorsed clinical genetic testing for DCM patients and families[2,3]
- Prevalence of BAG3 DCM in US is estimated to be as high as 30,000 patients[4] and is expected to grow with increasing genetic testing and disease awareness

Initial Proof-of-Concept for AAV9-BAG3 Supports Further Development

- Initial proof of concept for AAV9-BAG3 demonstrated in BAG3-knockout mouse model

Ejection fraction in WT and BAG3 +/- mice treated at age 6 to 8 weeks with AAV9-GFP or AAV9-BAG3



- Evaluating optimal development pathway; IND planned H1 2024

AAV, adeno-associated virus; BAG3, BCL2-associated athanogene 3; DCM, dilated cardiomyopathy; EF, ejection fraction; GFP, green fluorescent protein; H1, first half of the year; IND, investigational new drug; WT, wild type. Haploinsufficiency data published in Myers VD et. al, *J Cell Physiol.* 2018; AAV-BAG3 administration adapted from data published in Myers VD et. Al., *JAMA Cardiol.* 2018; and unpublished data from the Feldman lab.

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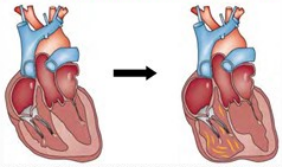
Project Pegasus (PKP2-ACM)

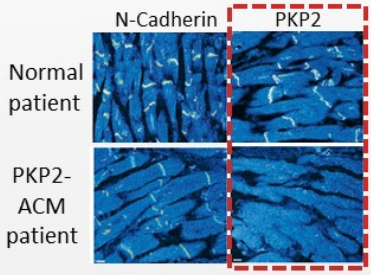
PKP2-Arrhythmogenic Cardiomyopathy (ACM)*:
A high-risk disease with no curative options

Advanced ACM Heart with fibrofatty replacement in right ventricle



Electrical manifestations can precede structural abnormalities

ACM: Diminished Myocardial PKP2



Disease Etiology

- Autosomal dominant mutations in *PKP2* gene, which encodes for Plakophilin-2, a component of the desmosome localized to cardiac intercalated discs

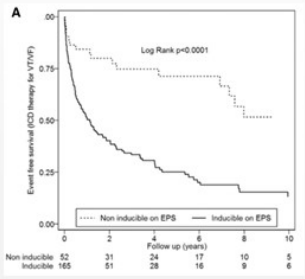
Therapeutic Challenges

- Current standard of care includes beta-blockers, anti-arrhythmic agents, and ablation
- Available treatments do not modify disease progression; no curative therapeutic options

Clinical Manifestations

- Mean age at presentation: 35y (±18) [1]
- 5-10% annual risk of sustained ventricular arrhythmias (VA), with higher risk in patients who present with disease symptoms (index patients)[2-3]
- Lifetime VA risk approximately 100% in index patients [4]
- ICD placement in >80% of index pts [5]
- For pts with ICDs:
  - 45-75% will have ICD firing (shock) over 3-5 years
  - ≥50% 2 year incidence of firing in subgroups:
    - male; • EPS-induced VT; • history of VT;
    - ≥3 ECG leads with TWI; • >1000 PVC/24h [5-6]

Kaplan-Meier Incidence of ICD Firing



Event free survival in ACM patients with ICDs based on VT inducibility on pre-ICD EPS study

- *>50% of patients who were inducible on EP study had an ICD firing over 2 year follow-up*

Estimated Prevalence (US+EU): ~50,000

Biopsy figure adapted from: Asimaki et al. NEJM, 2009; Table adapted from Dalal et al. Circulation 2006. SOC: standard of care; CM: cardiomyopathy; HF: heart failure: HTx: heart transplantation; RV: Right ventricular; SD: Standard Deviation; VT: ventricular tachycardia; LBBB: left bundle branch block; ICD: implantable cardioverter defibrillator; RVEF: right ventricular ejection fraction; LV: left ventricle; SVA: sustained ventricular arrhythmia.

*This cardiomyopathy initially manifests in the right ventricular free wall, so the disease was termed arrhythmogenic right ventricular dysplasia/cardiomyopathy (ARVD/ARVC). However, since left dominant and biventricular forms have also been observed, this has led more recently to the use of the term "ACM". 1. Bhonsale. EHJ 2015; 36: 847-55. 2. Towbin JA. Heart Rhythm 2019;16(11). 3. Cadrin-Tourigny J. Eur Heart J 2022;43. 4. Groenweg. Circ Cardiovasc Genet 2015; 8: 437-46. 5. Calkins. Circ 2017; 136: 2068-82. 6. Orgeron. J Am Heart Assoc 2017: e006242.

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PKP2-ACM Prevalence in the US and EU

ACM prevalence

1:1000 to 1:5000

Peters 2004, McKenna 2021

PKP2 variants

32.9%

2,572 ACM patients assessed from 13 publications an aggregated mean of **32.9% had *PKP2* mutations**[1]

ACM-PKP2 US & EU Prevalence

~50,000

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

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.
McKenna WJ, Judge DP. Epidemiology of the inherited cardiomyopathies. Nat Rev Cardiol. 2021;18(1):22-36.
[1] Data on file

Proof of Concept in Translationally Relevant Animal Model

Completed RCKT Studies with *Cardiomyocyte-specific PKP2 Knockout Mouse Model of ACM*

- Initial POC evaluated 4 AAV Vectors: Cardiac Functional & Structural Analyses
- Dose-related effects evaluated with 2 AAV vectors: Cardiac Functional & Structural Analyses
- Evaluated Survival, Functional, and Anatomic Benefit in 'Arrest Progression' Models
  - Including delivery of AAV *+7 or +14 Days after* induction of PKP2 knockout and subsequent disease onset

Analyses Include:

- Survival
- Echocardiography and ECG
- PKP2 expression (IF and WB)
- Cardiac pathology & fibrosis
- Vector DNA, transgene mRNA
- General safety including pathology

Ongoing sponsored research. No future royalty obligations

Academic Partner:

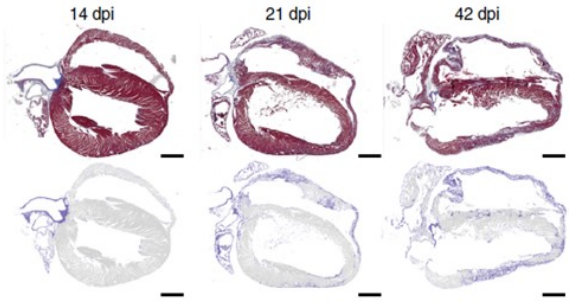
NYU Grossman School of Medicine

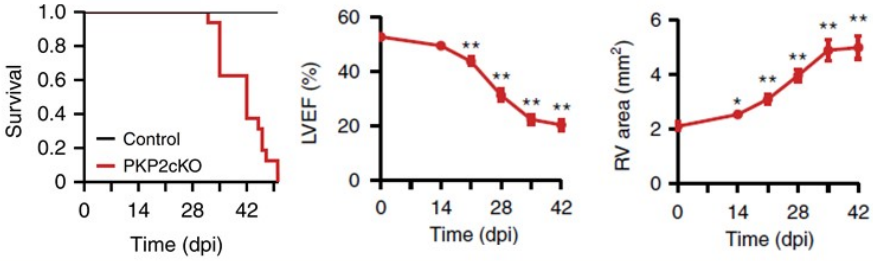
Mario Delmar, MD, PhD
Patricia and Robert Martinsen Professor of Cardiology, Department of Medicine; Division of Cardiology, NYU Grossman School of Medicine

Marina Cerrone, MD
Research Associate Professor, Co-Director, Inherited Arrhythmia Clinic, Department of Medicine; Division of Cardiology, NYU Grossman School of Medicine

Tamoxifen-induced ACM in the PKP2-cKO Mouse Model

- The PKP2-cKO mouse model recapitulates ACM following induction of PKP2 KO by tamoxifen (TAM) injection
- Progression of cardiomyopathy evidenced by Masson's trichrome staining of heart sections in PKP2-cKO mice from 14 to 42 days post-TAM (dpi)
- 100% mortality by day ~50 following TAM injection
- Left ventricular ejection fraction (LVEF) diminishes significantly across time
- Right ventricular (RV) enlargement occurs across time
- Premature Ventricular Contractions (PVCs) are a clinical hallmark of ACM and emerge in the animal model because of Pkp2 loss





Cerrone M, Montnach J, Lin X, et al. Plakophilin-2 is required for transcription of genes that control calcium cycling and cardiac rhythm. Nat Commun. 2017;8(1):106. Published 2017 Jul 24.
ISO, isoproterenol; cKO: conditional knockout

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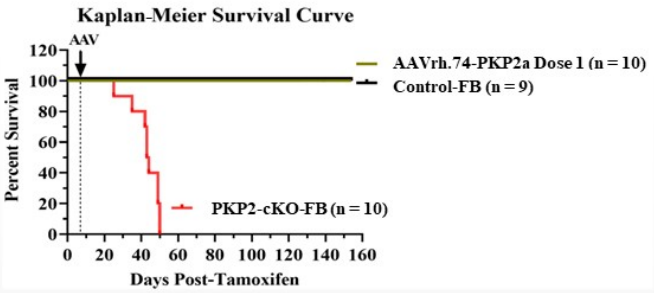
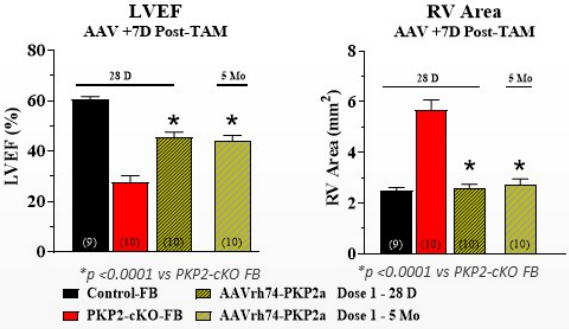
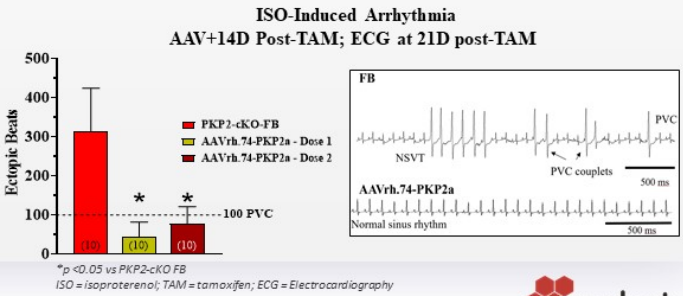
Increased Survival & Preserved Cardiac Function in the PKP2-cKO Model





- AAVrh.74-PKP2 delivered ***7 days post-TAM***:
  - **100% survival to 5 months,** compared to 100% mortality by day ~50 in PKP2-cKO control animals
  - **Preserved Ejection Fraction and Right Ventricular Area** at 28 Days, sustained to 5 months
- AAVrh.74-PKP2 delivered ***14 days post-TAM:***
  - Mitigated isoproterenol-induced PVCs and arrhythmia, disease-related characteristics of ACM
  - Robust benefit on survival, cardiac function & structure to 5 months[1]



*p <0.05 vs PKP2-cKO FB
ISO = isoproterenol; TAM = tamoxifen; ECG = Electrocardiography

[1]Data not illustrated in Figures

Optimal Gene Therapy for PKP2-ACM, Expected to be First-and Best-In-Class

cDNA/isoform:

- **PKP2a:** full wild type coding sequence of therapeutic gene, protein loss drives ACM

AAV Serotype:

- ***AAV.rh74* serotype associated with favorable safety profile in DMD/LGMD2E[1-2]; potential for safe administration at optimal doses for adult ACM patients**

Cardiac-Specific Promoter:

- Effectively drives expression of therapeutic transgene in cardiomyocytes; minimizes off-target effects

Route of Administration:

- **Intravenous (IV)** Pharmacology studies demonstrate efficient cardiac transduction with IV administration

Robust Proof of Concept in Disease Relevant Animal Model:

- **NYU Cardiac-specific cKO-PKP2 mouse** (biologically relevant translational model)

[1]Rodino-Klapac et. al. Safety, β-Sarcoglycan Expression, and Functional Outcomes From Systemic Gene Transfer of rAAVrh74.MHCK7.hSGCB in LGMD2E/R4. Presented at the Muscular Dystrophy Association (MDA) Conference. Nashville, TN, March 13–16, 2022.
[2]Mendell et. al. A Phase 2 clinical trial evaluating the safety and efficacy of delandistrogene moxeparvovec (SRP-9001) in patients with Duchenne muscular dystrophy. Presented at the 2022 Muscular Dystrophy Association (MDA) Conference Nashville, TN, March 13–16, 2022.

Clinical Development Plan

Moving Toward Phase 1 Dose Escalation Study

Completed or Ongoing Activities

- ✓ GMP drug product manufacturing completed
- ✓ Pharmacology and GLP toxicology studies
- ✓ Potency assay
- ✓ Upcoming Scientific Advisory Board
- Clinical trial planning activities, including site selection, underway
- Submit Orphan Disease Designation
- FDA IND submission anticipated **Q2 2023**

High Level Phase 1 Proposed Trial Design

- Study design:
  - FIH, multi-center, 3+3 dose escalation[a] study
  - Two dose levels planned
- Target population:
  - High risk adult PKP2-ACM patients
- Primary endpoint:
  - Safety events related to RP-A601
- Secondary and exploratory endpoints: TBD

Natural History

- To provide context for the Phase 1 study results, we will leverage data from existing ACM registries as well as longitudinal and population-level data from published case series

FDA, Food and Drug Administration; 1H, first half of the year. FIH, first in human.
[a] potential for up to 3 additional patients per cohort in setting of DLTs in 1st 3 pts

Rocket – The Leader in Rare Disease Gene Therapy

- ✓ Pre-eminent maturing gene therapy pipeline in which each program is First- and Best-in-Class
- ✓ Experienced management team with a history of delivering transformative and curative therapies to patients with devastating diseases
- ✓ Well-capitalized and poised to elevate from a clinical-stage to a commercial-stage company

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THANK YOU!

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Exhibit 99.2

Rocket Pharmaceuticals Expands Cardiac Gene Therapy Portfolio with Addition of RP-A601 for PKP2-ACM and Announces Positive Updated Phase 1 Data for RP-A501 in Danon Disease

RP-A601 for Arrhythmogenic Cardiomyopathy due to PKP2 pathogenic variants (PKP2-ACM) represents meaningful commercial opportunity with estimated prevalence of 50,000 adults and children in the U.S. and EU; IND filing anticipated in Q2 2023

In support of Phase 2 pivotal study, latest positive Phase 1 data in Danon Disease recently shared with FDA demonstrate improvements in all biomarker and clinical endpoints across pediatric and adult patients with marked difference vs. natural history; two cGMP manufacturing runs completed with high product quality at in-house facility

BLA filings for LAD-I and FA on track for Q2 2023 and Q4 2023, respectively

Well-capitalized to develop full pipeline of assets with $401M (preliminary, unaudited) in cash and cash equivalents; operational runway now expected through 2024 (inclusive of PKP2-ACM program)

CRANBURY, N.J. – **Jan. 9, 2023** – Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a leading late-stage biotechnology company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders with high unmet need, today announces the addition of RP-A601 to Rocket's cardiac gene therapy portfolio as well as anticipated highlights for the year ahead across the Company's world-class pipeline of lentiviral and AAV gene therapy programs targeting rare hematologic and cardiovascular diseases. These announcements and anticipated highlights will be presented at the 41st Annual J.P. Morgan Healthcare Conference today at 2:15 p.m. PT by Gaurav Shah, M.D., Chief Executive Officer, Rocket Pharma.

"I am extremely excited to build on our significant progress in 2022 as we advance six programs with compelling clinical and/or preclinical proof of concept, including the addition of RP-A601 for the treatment of arrhythmogenic cardiomyopathy due to plakophilin 2 pathogenic variants (PKP2-ACM), expanding our industry-leading cardiovascular AAV gene therapy portfolio. Equally, we look forward to submitting our first regulatory filings, for Fanconi Anemia and Leukocyte Adhesion Deficiency (LAD-I), this year," said Dr. Shah. "RP-A601 for ACM aims to address the unmet needs of approximately 50,000 adults and children in the U.S. and EU who face this devastating genetic heart disease marked by arrhythmias and heart failure that often results in sudden cardiac death. We are moving this program forward into the clinic having demonstrated robust preclinical proof of concept using an rh74 serotype that has been associated with a favorable safety profile in the clinic for other diseases. We anticipate being first to clinic with a submission of an IND for RP-A601 in the second quarter of 2023 for a multi-center, dose-escalation study for the treatment of PKP2-ACM."

Dr. Shah continued, “Additionally, positive data updates today in Danon Disease include several months of additional Phase 1 results that demonstrate further robust improvements in all biomarkers, and in how both adult and pediatric patients function and feel, that diverge meaningfully from natural history patients of similar age and disease characteristics. We have shared these updates with the FDA as part of our pivotal Phase 2 study design discussions. Additionally, we have successfully produced two Danon AAV cGMP batches at our Cranbury, N.J. facility with improved product specifications versus Phase 1 thus taking another essential step towards commercial readiness.”

“Clinical programs from our hematology portfolio, represented by our Phase 2 pivotal studies in Fanconi Anemia and LAD-I, remain on track. We are laser focused on regulatory submissions, with BLA filings anticipated for LAD-I and FA in the second quarter and fourth quarter of 2023, respectively,” said Dr. Shah. “Lastly, I am pleased to note an extension of our cash runway, which we expect to fund operations through 2024. I am incredibly pleased with our progress, and excited about the catalyst-rich year ahead as we continue on our path to becoming a fully integrated rare disease and gene therapy company with capabilities extending from discovery through manufacturing and commercialization.”

RP-A601 for PKP2 Arrhythmogenic Cardiomyopathy on Track for IND Submission in Q2 2023

- ACM, or arrhythmogenic right ventricular dysplasia (ARVC), due to plakophilin 2 pathogenic variants (PKP2-ACM), is a high-risk cardiomyopathy caused by autosomal dominant mutations in the *PKP2* gene. ACM is characterized by frequent and life-threatening ventricular arrhythmias and structural ventricular myopathy. Available treatments fail to address the underlying genetics and disease biology and do not alter disease progression. PKP2-ACM affects approximately 50,000 people in the U.S. and EU.
- Preclinical proof of concept from a translationally relevant animal model has been demonstrated following Rocket-sponsored studies with academic partners at NYU Grossman School of Medicine. The preclinical studies with a cardiomyocyte-specific PKP2 knockout mouse model of ACM evaluated initial proof of concept and dose-related effects of AAV vectors, including survival, functional and anatomic benefits. Notably, studies evaluated the delivery of AAV at seven and 14 days following induction of PKP2 knockout and subsequent disease onset.
- Results demonstrated increased survival and preserved cardiac function in the PKP2 knockout mouse model.
    - 100% of adult PKP2 knockout mice receiving RP-A601 seven days after knockout induction demonstrated survival to the five-month duration of the evaluation compared to 100% mortality by approximately day 50 in PKP2 knockout mice receiving formulation control. PKP2 knockout mice receiving RP-A601 were observed with preserved ejection fraction and right ventricular area at 28 days, sustained to five months.
    - Fourteen days following RP-A601 administration, PKP2 knockout mice demonstrated robust survival with a similar degree of cardiac benefit to five months. RP-A601 was also associated with mitigation of isoproterenol-induced PVCs and arrhythmias, which are major morbidity components of ACM.
- GMP drug product manufacturing is completed, and a potency assay has been developed. Based on the strength of the pharmacology and toxicology data, Rocket anticipates filing an IND in the second quarter of 2023 for a Phase 1 multi-center, dose-escalation study evaluating two doses.

RP-A501 for Danon Disease Moving Toward Global Phase 2 Pivotal Study

- Efficacy results from the Phase 1 study continue to demonstrate durable improvement or stabilization of clinical parameters in the Danon Disease patients treated to date. Notably, these patients' improvements and stabilization of brain natriuretic peptide (BNP) and New York Heart Association (NYHA) class are in stark contrast to BNP increases and NYHA class deterioration observed in a representative sample of pediatric and adolescent natural history patients. These data have been presented recently to the FDA.
- The Phase 2 pivotal trial remains on track for initiation in the second quarter of 2023 based on ongoing and productive FDA interactions.
- Robust Technical Operations capabilities are highlighted by the advancement of in-house AAV cGMP manufacturing at Rocket's state-of-the-art facility in Cranbury, N.J. Completion of two in-house production runs resulted in high-quality drug substance enabling an approximately threefold increase in the number of patients treatable per batch, a significantly improved full versus empty particle ratio, and promising product comparability data generated to date compared to the Phase 1 material manufactured externally.

J.P. Morgan Healthcare Conference Webcast

Gaurav Shah, M.D., Chief Executive Officer, Rocket Pharma, will be presenting at the 41st Annual J.P. Morgan Healthcare Conference today at 2:15 p.m. PT. A live audio webcast of the presentation is available under "Events" in the Investors section of the Company's website at https://ir.rocketpharma.com. The webcast replay will be available on the Rocket website following the conference.

Anticipated 2023 Milestones

Hematology (LV)

RP-L102 for Fanconi Anemia

- Product filing – Q4 2023
- Complementation Groups C & G Investigational New Drug (IND) submission – 2024

RP-L201 for Leukocyte Adhesion Deficiency-I (LAD-I)

- Product filing – Q2 2023
- LAD-I moderate study initiation – Q4 2023

RP-L301 for Pyruvate Kinase Deficiency (PKD)

- Phase 2 pivotal study initiation – Q4 2023

LV Platform Enhancements

- Non-genotoxic conditioning for LV – 2024

Cardiovascular (AAV)

RP-A501 for Danon Disease

- Completed two in-house cGMP batches – Q1 2023

- Planned Phase 2 pivotal study initiation – Q2 2023
- EU Investigational Medicinal Product Dossier (IMPD) filing – Q3 2023
- Danon female study initiation – Q4 2023

RP-A601 for PKP2-ACM
- IND filing – Q2 2023

BAG3-Associated DCM
- IND filing – 2024

Undisclosed Candidates
- Disclosure of additional Wave 2 assets – 2024

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 preclinical AAV-based gene therapy programs in PKP2-arrhythmogenic cardiomyopathy (ACM) and 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 its anticipated 2023 and 2024 milestones, goals and target, ability to develop its full pipeline of assets with its current cash and cash equivalents, the sufficiency of its cash and cash equivalents to fund operations through 2024, guidance for 2023 and 2024 in light of COVID-19, the safety and effectiveness of product candidates that Rocket is developing to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD), Danon Disease (DD) and other diseases, 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, our expectations regarding the delays and impact of COVID-19 on clinical sites, 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, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, and 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, 2021, filed February 28, 2022 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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