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SEEKING GENE THERAPY CURES

DISCLAIMER

Various statements in this release concerning Rocket's future expectations, plans and prospects, including without limitation, Rocket's expectations regarding its guidance for 2022 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), and Danon Disease, 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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ABOUT ROCKET PHARMACEUTICALS

"For the first time in history, **we are discussing not just effective treatments but potential cures at the genetic level**, which is the deepest essence of who we are as physical beings."

— GAURAV SHAH, MD | CEO

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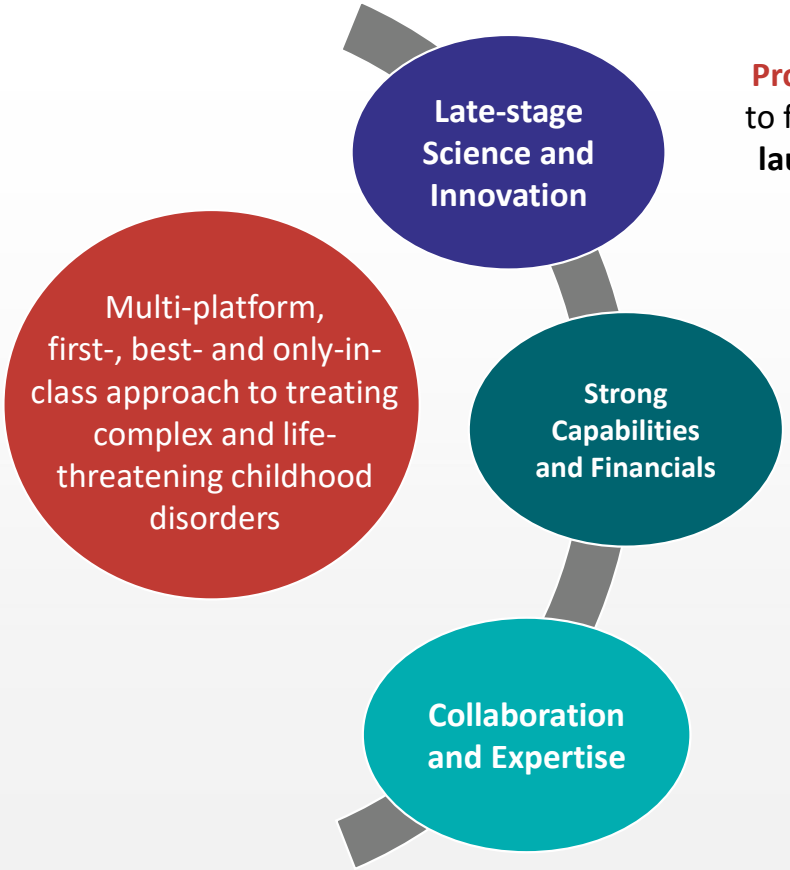
Vision: Seeking Gene Therapy Cures

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 & European registration & 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 ft²
US-based in-house facility dedicated to AAV cGMP manufacturing

$321M
in cash and cash equivalents

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

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

Expert Leadership With Proven Track Record

Gaurav Shah, M.D.
Chief Executive Officer
Spearheaded Kymriah (CART-19) development at Novartis towards approval

Kinnari Patel, Pharm.D., MBA
President and Chief Operating Officer
Led Opdivo and six rare disease indication approvals

Mayo Pujols
Chief Technical Officer, EVP
~30 years technical operations and GMP manufacturing expertise

Isabel Carmona, J.D.
Chief Human Resources Officer, SVP
Seasoned leader in human resources, legal and compliance across life sciences, financial services and IT

Carlos Martin, BA, MBA
Chief Commercial Officer, SVP
15+ years global & local leadership, commercial strategy and new product launches

Raj Prabhakar, MBA
Chief Business Officer, SVP
~20 years cell, gene and biotech business development

Gayatri R. Rao, M.D., J.D.
Chief Development Officer of LV, SVP
7-Year former Director of FDA's Office of Orphan Products Development

Jonathan Schwartz, M.D.
Chief Medical Officer, SVP
Led multiple biologics approvals

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

Jessie Yeung, MBA
Investor Relations & Corporate Finance, VP
15+ years investor relations, corporate finance and capital market experience

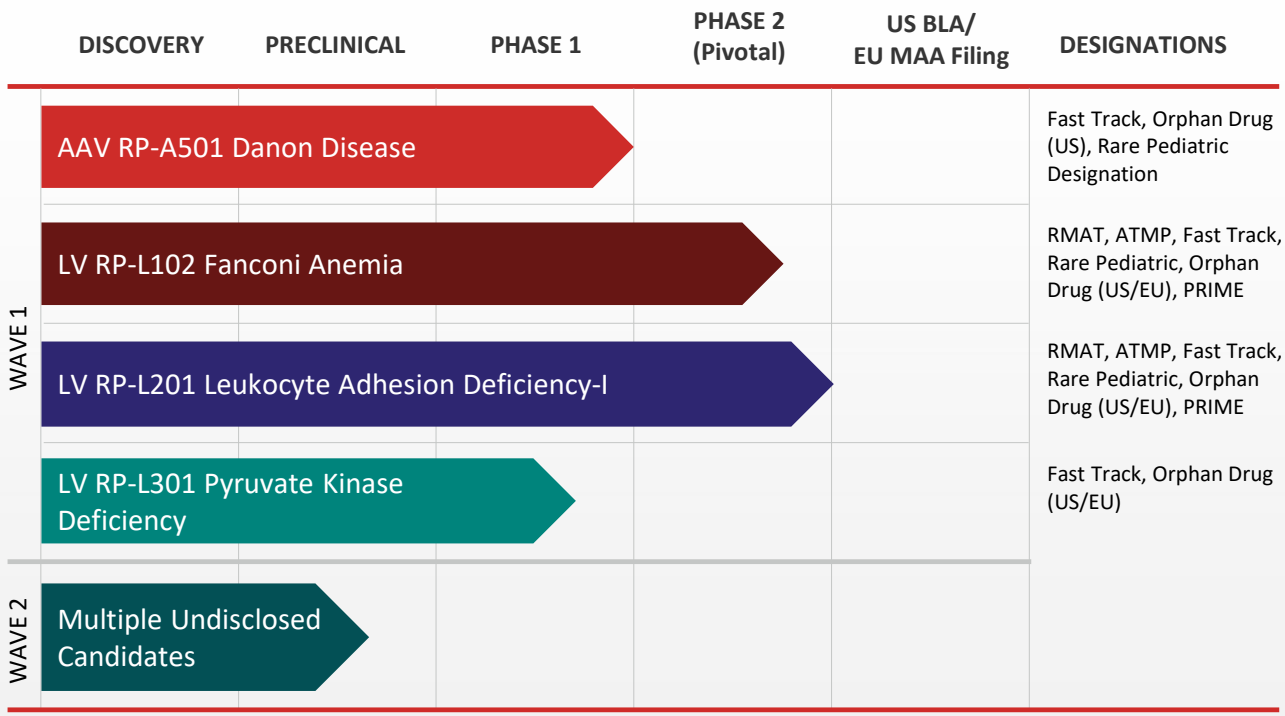
Peggy Speight
Head of Quality Assurance, VP
20+ years quality assurance and regulatory compliance expertise gained in pharma and at FDA

Strong Science, Carefully-selected Assets and Smart Execution:
Four Programs With Compelling Clinical POC

Criteria used to select programs

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

Four programs with compelling clinical proof of concept



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.
Data on file. Rocket Pharmaceuticals. 2022.

Developing **First-, Best- and Only-in-Class** Therapies for Rare Diseases With Extensive Unmet Needs

Strong science, carefully-selected assets and smart execution

- Right technology for the target
- Clean MOAs: correct proteins are made in correct cells for disorders caused by single gene mutations
- Well-defined, achievable endpoints
- In-house AAV cGMP manufacturing with capabilities to support commercial products and scaling

Proven management expertise

- Strong drug development track record, successful BLA filings
- Engagement with health authorities to outline a predictable review pathway
- HEOR work to inform value-based pricing strategy
- Creation of “go-to commercial” infrastructure

Near term inflection points drive value

2022 Q2

- ✓ Achieve in-house AAV (cGMP) readiness
- ✓ **LAD-I**: Pivotal Phase 2 data
- ✓ **FA**: Phase 2 primary endpoint readout

Q3

- **Danon**: Phase 1 pediatric cohort data

Q4

- **Danon**: Initiate Phase 2 pivotal trial activities
- **FA**: Potential guidance on BLA filing timeline
- **PKD**: Phase 1 data

2023

- **LAD-I, FA**: BLA/MAA filings
- **PKD**: Initiate Phase 2 pivotal trial activities
- Wave 2 pipeline enters clinic

AAV, adeno-associated virus; BLA, Biologics License Application; cGMP, current Good Manufacturing Processes; FA, Fanconi Anemia; H1, first half of the year; HEOR, health, economics and outcomes research; LAD-I, Leukocyte Adhesion Deficiency-I; MOA, mechanism of action; PKD, Pyruvate Kinase Deficiency; Q2, second quarter of the year; Q3, third quarter of the year; Q4, fourth quarter of the year.
Data on file. Rocket Pharmaceuticals. 2022.

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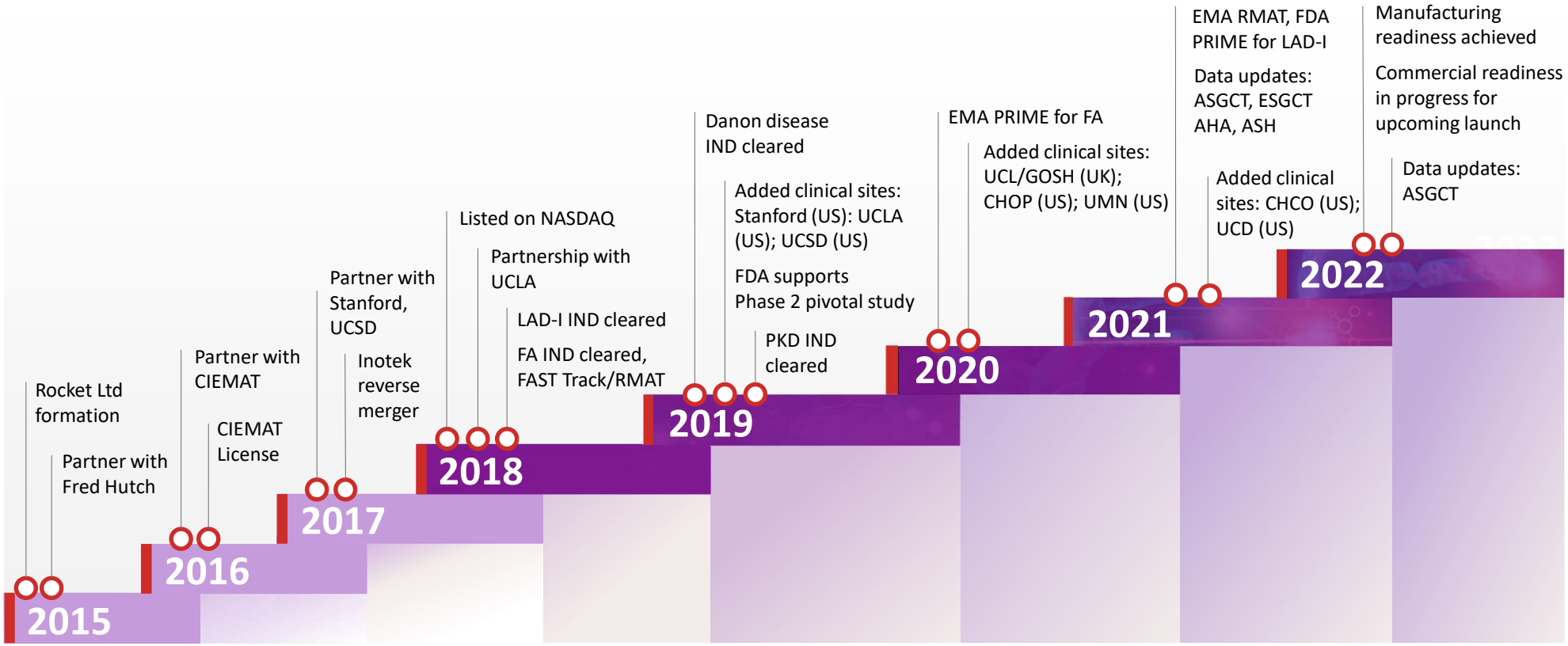
Strategically Building a Leading Gene Therapy Company



ASGCT, American Society of Gene & Cell Therapy; ASH, American Society of Hematology; CHCO, Children's Hospital of Colorado; CHOP, Children's Hospital of Philadelphia; CIEMAT, Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas; EMA, European Medicines Agency; ESGCT, European Society for Gene and Cell Therapy; FA, Fanconi Anemia; FDA, Food and Drug Administration; IND, Investigational New Drug; LAD-I, Leukocyte Adhesion Deficiency-I; PKD, Pyruvate Kinase Deficiency; PRIME, PRIority MEdicines; RMAT, Regenerative Medicine Advanced Therapy; UCLA, University of California, Los Angeles; UCSD, University of California San Diego; UCD, University of Colorado, Denver; UMN, University of Minnesota. Data on file. Rocket Pharmaceuticals. 2022.

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Strong, Strategic Approach to **Gene Therapy Manufacturing**

In-house capabilities

AAV cGMP
manufacturing with capabilities to support commercial products and scaling

Process Development, Analytics and QC testing

Streamlined manufacturing capabilities to allow for **cost-effective commercialization**

~100,000 ft²
facility in Cranbury, NJ

AAV, adeno-associated virus; cGMP, current Good Manufacturing Processes.
Data on file. Rocket Pharmaceuticals. 2022.

World-class **Scientific Experts** and Partners

Ciemat

UCL

UCLA

FRED HUTCH

Children's Hospital of Philadelphia

IIS FJD INSTITUTO DE INVESTIGACIÓN SANITARIA FUNDACIÓN JIMÉNEZ DÍAZ

NHS Great Ormond Street Hospital for Children NHS Foundation Trust

REGENXBIO

University of Colorado

Penn

Stanford Medicine

Hospital Infantil Universitario Niño Jesús

UC San Diego

University of Minnesota

Fundación Jiménez Díaz

CIRM California's Stem Cell Agency

ciber

Data on file. Rocket Pharmaceuticals. 2022.

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UNMET NEEDS AND MARKET

“Caring for someone with Danon, while you, yourself have Danon is very hard. Most days we are at clinic appointments or having a procedure done to check on our hearts. The other times we are at home dealing with chest pain, rapid heart rates, muscle pains and learning issues in school. With each new day we have a renewed hope that with time and clinical trials we will be able to someday cure this rare and deadly disease."

— DANON DISEASE PATIENT AND MOTHER OF TWO BOYS LIVING WITH DANON

“We never went through the bone marrow transplant route and only had to deal with cancer and the complications associated with chemotherapy and radiation therapies. We lost two children…to this awful condition. May future research yield positive outcomes.”

— FATHER OF TWO CHILDREN WITH FANCONI ANEMIA

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Rare Diseases Are Associated With a Reduced Lifespan[1]

400 million
people globally are affected by a rare disease[1]

Children account for
50%
of rare disease patients[1]

80% **of rare diseases** have monogenic origins[1]

3 of 10 children with a rare disease die before their fifth birthday[1]

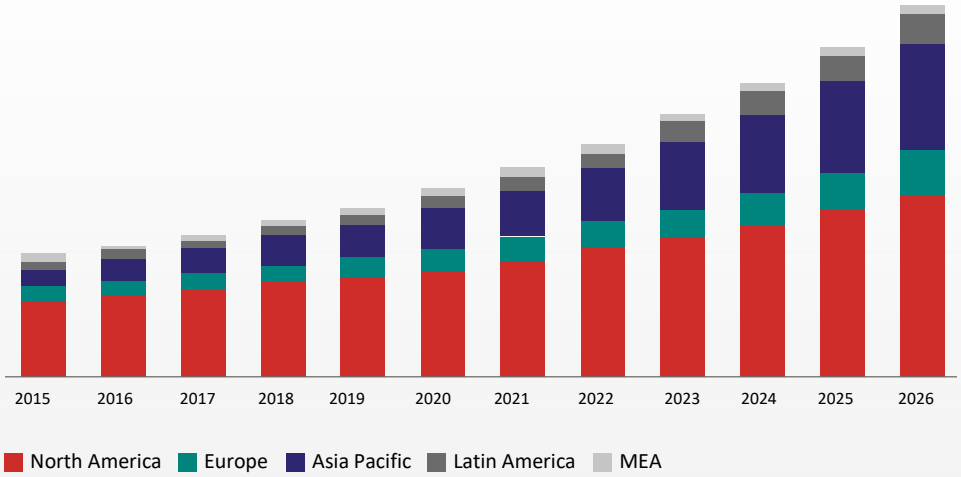
Only about 5% of rare diseases have an FDA-approved drug treatment[1]

FDA, Food and Drug Administration.

1. Global Genes. Accessed April 2022. https://globalgenes.org/rare-disease-facts/

Market for Rare Disease Treatment Is **Rising**

Rare disease treatment market by region, 2015-2026 (USD million)[1]



Rare disease treatment market by drug type, 2019 (USD million)[1]

- Rare disease treatment market is projected to grow from **$161.4 billion in 2020** to **$547.5 billion by 2030**[2]
- CAGR of 13.1% projected by 2030[2]

Orphan drug approvals have increased

4-fold[3]

CAGR, compound annual growth rate; CDER, Center for Drug Evaluation and Research; MEA, Middle East and Africa.

1. Global Market Insights. Accessed April 2022. https://www.gminsights.com/industry-analysis/rare-disease-treatment-market **2.** Global News Wire. Accessed August 2022. https://www.globenewswire.com/en/news-release/2021/02/24/2181634/0/en/Global-Rare-Disease-Market-is-estimated-to-be-US-547-5-billion-by-2030-with-a-CAGR-of-13-1-during-the-forecast-period-by-PMI.html **3.** AHIP. Accessed April 2022. https://www.ahip.org/how-big-pharma-makes-big-profits-on-orphan-drugs

Costs Associated With Rare Diseases Have Increased Exponentially[1]

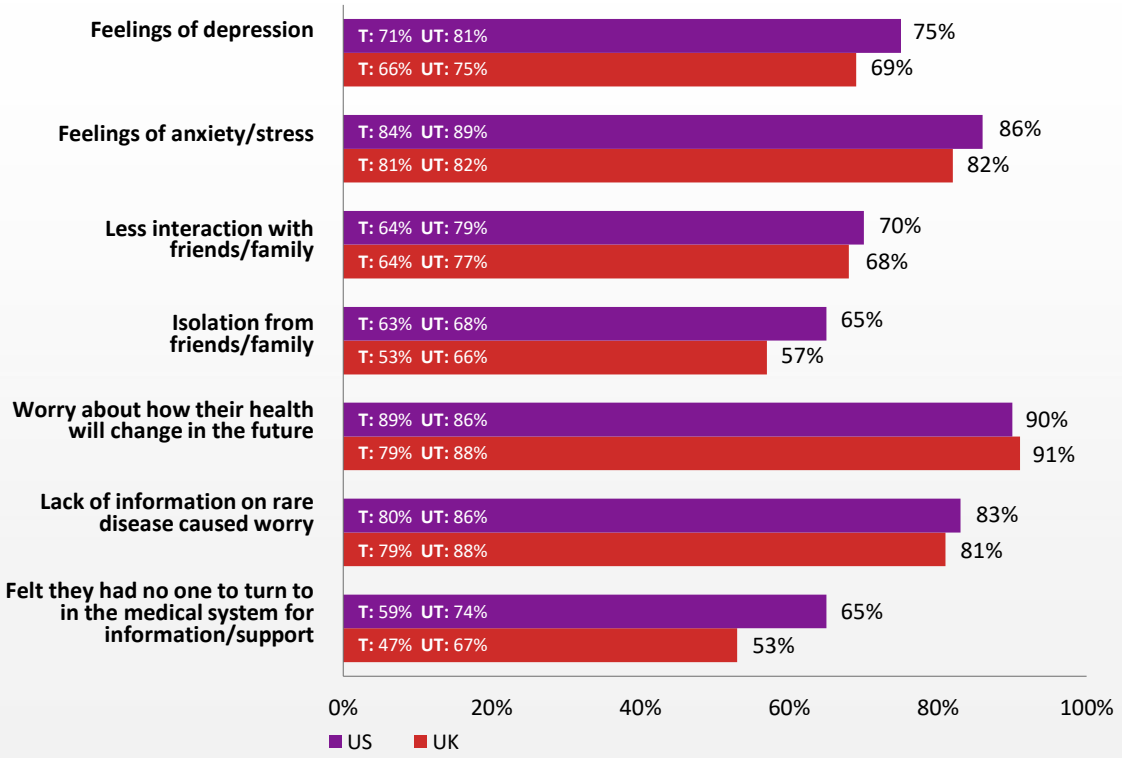
Economic impact[1]

26-fold increase in average per-patient annual cost for orphan drugs* compared to doubled costs for specialty and traditional drugs[1]

Patients with rare diseases or their **caregivers** are **often compelled to leave the workforce**[2]

Cost of **bone marrow** and **heart transplants** range between **$600K** and **$1.5M** respectively, plus **$50k** to **150K** annually in associated costs[3]

Emotional impact[4]



*An orphan drug is a pharmaceutical agent developed to treat medical conditions, which, because they are so rare, would not be profitable to produce without government assistance.
T, treatable; UT, untreatable.

1. AHIP. Accessed April 2022. https://www.ahip.org/news/press-releases/drug-prices-for-rare-diseases-skyrocket-while-big-pharma-makes-record-profits **2.** Every Life Foundation for Rare Diseases. Accessed April 2022. https://everylifefoundation.org/wp-content/uploads/2021/02/The_National_Economic_Burden_of_Rare_Disease_Study_Summary_Report_February_2021.pdf **3.** Data on file. Rocket Pharmaceuticals. 2022. **4.** Global Genes. Accessed April 2022. https://globalgenes.org/wp-content/uploads/2013/04/ShireReport-1.pdf

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PIONEERING GENE THERAPY CLINICAL PROGRAMS

"Due to the high unmet need, there is significant interest within the FA community from both patients and health care providers for an alternative low-toxicity therapy to address and, more specifically, prevent BMF. Overall, the investigational gene therapy – administered with a preventative intent and requiring no cytotoxic conditioning therapy – represents a compelling potential option for FA patients, even though this approach requires a more protracted time interval (i.e., 1-3 years) for recognition of phenotypic, genetic, and hematologic correction, relative to allogeneic HSCT."

— PRINCIPAL INVESTIGATOR OF ROCKET'S FA PROGRAM

"During the kids' entire childhood they had multiple infections – 'you name it they had it' – and were admitted to the hospital several times due to these infections. Since treatment, the kids are back in day care and have scraped their knees – but unlike their experience before gene therapy, this has not resulted in infections. This therapy "saved their lives" and without it don't know whether or not the kids would be alive at present. The therapy gave hope and hope that it will be available for other kids with severe LAD-I."

— FATHER OF THREE CHILDREN WITH SEVERE LAD-I

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

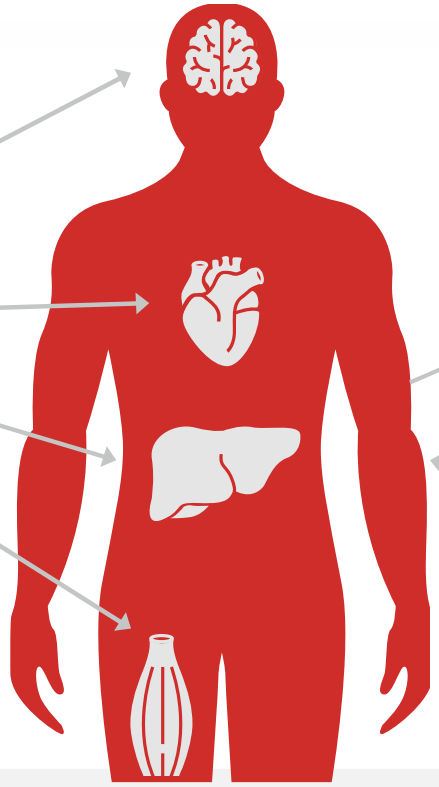
IN VIVO platform

Laboratory-produced AAV

Therapeutic AAV

Direct intravenous injection

RP-A501: Danon Disease



EX VIVO platform

Remove cells and isolate patient HSCs

Laboratory-produced LV

Therapeutic LV

Infusion of modified HSCs

Gene-modified HSCs

RP-L102: Fanconi Anemia

RP-L201: Leukocyte Adhesion Deficiency-I

RP-L301: Pyruvate Kinase Deficiency

AAV, Adeno-Associated Virus; LV, Lentiviral Vector.
Data on file. Rocket Pharmaceuticals. 2022.

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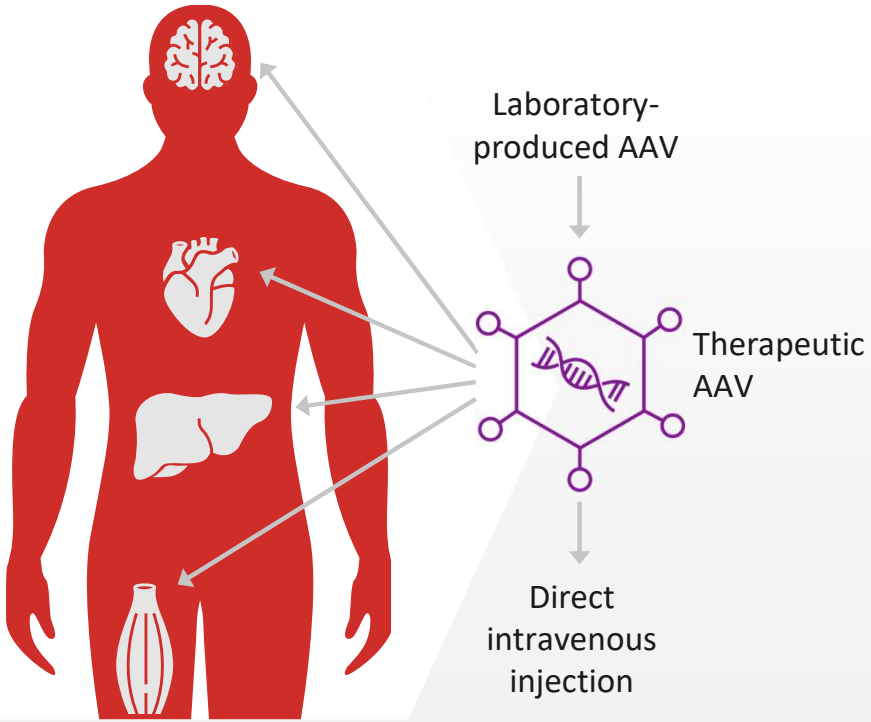
In Vivo Platform: Adeno-associated Virus (AAV)

IN VIVO (inside the body) AAV gene therapy



DANON DISEASE

Multi-system disorder with severe cardiomyopathy

- Transduction of non-dividing, terminally differentiated cardiomyocytes
- AAV9 serotype has been shown to have a particular propensity for cardiomyocytes
- rAAV9-vector DNA expresses *LAMP2B* gene
- Long-term durable expression anticipated because cardiomyocytes have minimal cell turnover

IDEAL FOR

AAV platform ideal for disorders that affect the heart, liver, eye or central nervous system

GOAL

Express an adequate quantity of normal protein to normalize cardiomyocyte structure and function

*Different AAV serotypes differ in their tropism, or the types of cells they infect, making AAV a very useful system for preferentially transducing specific cell types.
AVV, Adeno-Associated Virus; LAMP2B, Lysosome-Associated Membrane Protein 2B; rAAV9 , Recombinant Adeno-Associated Virus Serotype 9
Data on file. Rocket Pharmaceuticals. 2022.

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RP-A501 for Danon Disease: ***LAMP2B*** Gene Mutation

Disease etiology

- **LAMP2 mutation**
- **Autosomal dominant, monogenic X-linked disease**

Therapeutic challenges

- **Standard of care:**
  - Heart transplant
- **Limitations:**
  - Considerable morbidity and mortality
  - Not curative
  - Available to ~20% of patients

Cellular pathology

- **Impaired autophagy**
  - Prominent autophagic vacuoles
  - Myocardial disarray

Clinical manifestations

- **Severe cardiomyopathy**
  - Mortality secondary to heart failure or arrhythmia
  - Males: Aggressive disease course, median overall survival: 19 years
  - Females: Delayed presentation (~20 years) due to additional X chromosome; highly morbid & fatal disorder
- **Other clinical manifestations**
  - Skeletal myopathy
  - CNS manifestations
  - Ophthalmologic manifestations

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

CNS, central nervous system; LAMP2B, lysosome-associated membrane protein 2B; MOA, mechanism of action.
Boucek D et al. *Genet Med.* 2011;13(6):563-568.

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RP-A501: Danon Disease

First AAV Program in History to Address Monogenic Cardiomyopathy

Description

Recombinant AAV9 containing the human *LAMP2B* transgene

Clinical study

N=7 (Phase 1)

Primary endpoints:

- Safety
- Cardiomyocyte transduction, LAMP2B protein expression, histologic normalization
- Clinical stabilization or improvement

Selected secondary endpoints:

- Sustained stabilization or improvement in CV pathophysiology
- Sustained stabilization or improvement in echocardiographic, serologic and other clinical parameters of heart failure
- Overall survival

Key efficacy data

Efficacy in low-dose adult cohort:

- Robust LAMP2B cardiac expression
- Decreased BNP
- Improved ventricular wall thickness
- Improved NYHA class
- Stable or improved cardiac function

Safety

- Manageable safety profile
- Enhanced immunomodulatory regimen for pediatric cohort associated with:
  - Limited side effects
  - Mitigation of AEs observed in low- and high-dose adult cohorts

6MWT, 6-minute walk test; AVV, adeno-associated virus; BNP, B-type natriuretic peptide; CV, cardiovascular; LAMP2B, lysosome-associated membrane protein 2B; NYHA, New York Heart Association.
Data on file. Rocket Pharmaceuticals. 2022.

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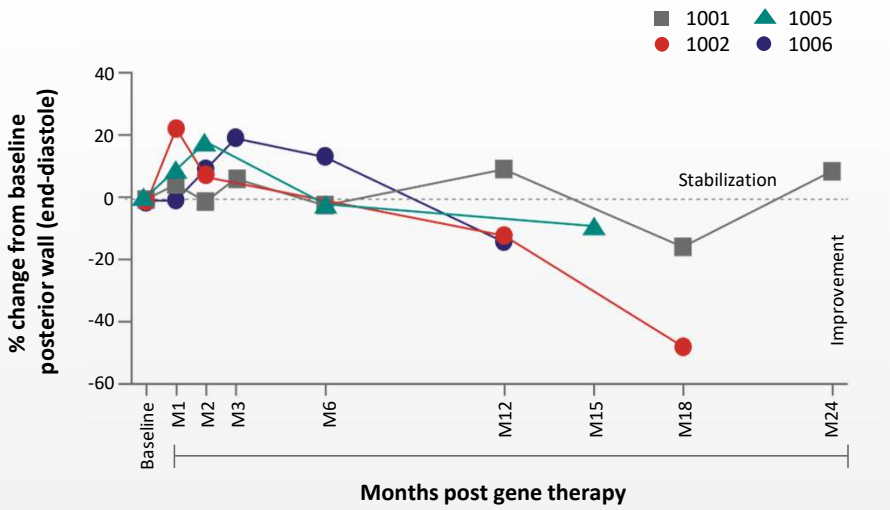
Reduction in Heart Wall Thickness Indicates Cardiac Remodeling

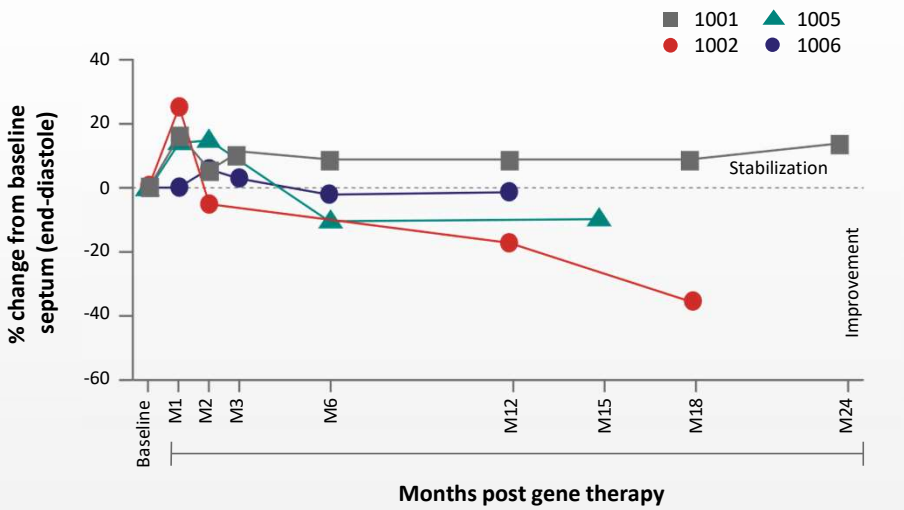
Treated patients show stabilization or improvement in LV wall and septal wall thickness compared to untreated† males with Danon disease

Posterior wall thickness in RP-A501–treated patients*



Danon Disease Natural History: LV posterior wall thickens by 0.74 ± 0.12 mm/year in untreated Danon males

Septal thickness in RP-A501–treated patients*



Danon Disease Natural History: septal wall thickens by 0.92 ± 0.15 mm/year in untreated Danon males*

*All echocardiographic parameters from local laboratory assessment; posterior wall: LVPWd, Septal wall: IVS. IVS, interventricular septum; LV; left ventricular; LVPWd, left ventricular posterior wall end diastole.
Data on file. Rocket Pharmaceuticals. 2022.

†Unpublished data from International Danon Disease Registry (not pictured on current slide).

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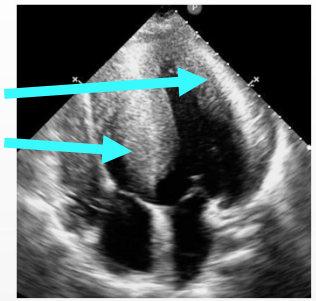
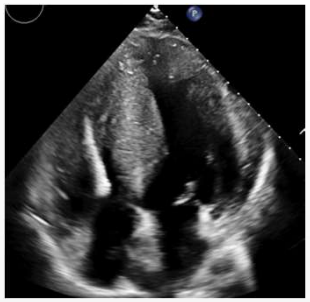
Reduction in Heart Wall Thickness Indicates Cardiac Remodeling

Baseline



Month 15



Representative images from patient 1005

All echocardiographic parameters are from local laboratory assessment by a single reader.

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Improved Cardiac Function Across Dose Levels

Stabilization or improvement of cardiac biomarkers and functional status is seen across treatment cohorts

Cohort	Patient ID	Age at enrollment (years)	Variable	Baseline	Most recent follow-up	Time of follow-up (months)
Adult – Low dose	1001*	17.5	NYHA class	II	II	24
			BNP (pg/mL)	70	30	
			6MWT (meters)	443	467	
	1002	20.4	NYHA class	II	I	18
			BNP (pg/mL)	942	200	
			6MWT (meters)	405	410	
	1005	18.3	NYHA class	II	I	15
			BNP (pg/mL)	176	44	
			6MWT (meters)	427	435	
Adult – High dose**	1006	21.1	NYHA class	II	I	12
			BNP (pg/mL)	123	41	
			6MWT (meters)	436	492	

*Corticosteroid compliance not closely monitored in initial patient.
6MWT, 6-minute walk test; BNP, brain natriuretic peptide; NYHA, New York Heart Association.
Data on file. Rocket Pharmaceuticals. 2022.

**Patient 1007 underwent heart transplant at 5 months for progressive Danon Disease, thus no subsequent data reported

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Improved Protein Expression Across Dose Levels

Endomyocardial LAMP2B protein expression is seen across dose levels

Cohort	Patient ID	LAMP2B protein expression (by IHC)* Month 12	LAMP2B protein expression (by Western Blot) Month 5-18
Adult – Low dose	1001†	2.5% (previously <15%)[a]	17.9%[d]
	1002	67.8%	21.2%[e]
	1005	92.4%[b]	61.1%[f]
Adult – High dose	1006	100%	18.2%[d]
	1007	100%[c]	RV: 45.1%[g] LV: 44.0%[g]

[a]Previously disclosed as a range due to high variance, now clarified.
[b]Month 9 data.
[c]Explant sample at Month 5.
[d]Month 6 data; inadequate sample at Month 12.
[e]Month 18 data; inadequate sample at Month 12.
[f]Month 9 data.
[g]Explanted heart; Month 5 data.

*Endomyocardial biopsies stained for LAMP2 compared to normal control samples. Percent area of cell staining was quantitated using software in a blinded fashion from 2 to 14 sections.
†Patient 1001 was only locally monitored for compliance for two weeks; longer compliance monitoring initiated after 1001 Qualitative assessment reported for samples with high variance.
IHC, immunohistochemistry; LAMP2, Lysosomal Associated Membrane Protein 2; LAMP2B, lysosome-associated membrane protein 2B; LV, left ventricle; RV, right ventricle.
Data on file. Rocket Pharmaceuticals. 2022.

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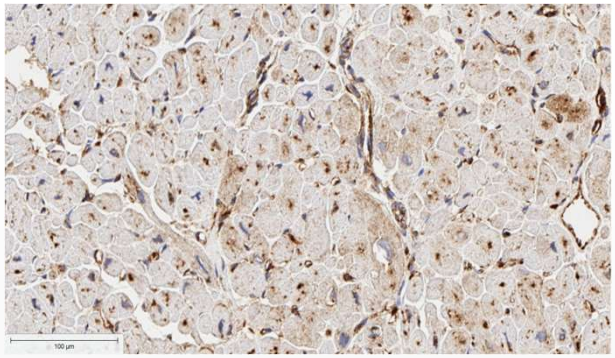
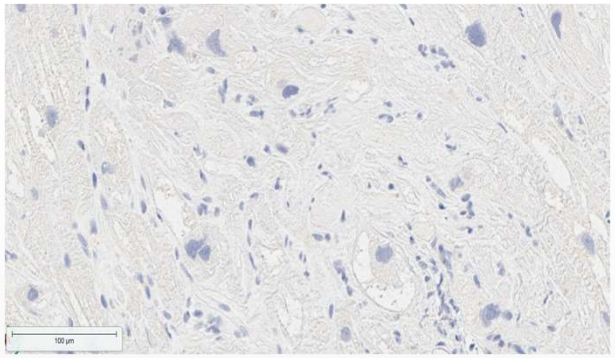
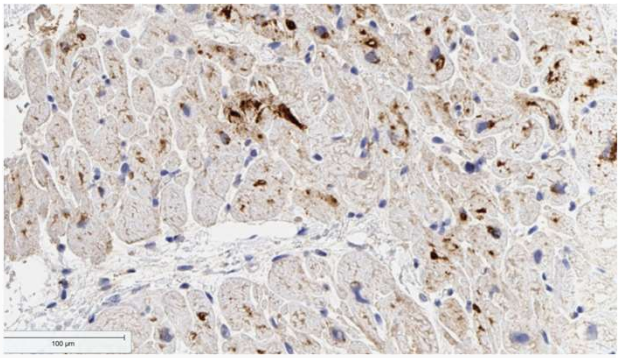
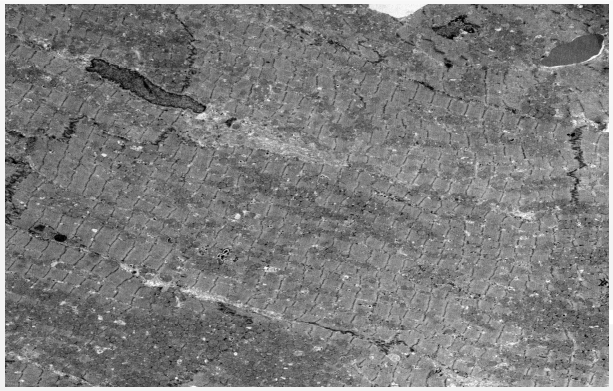
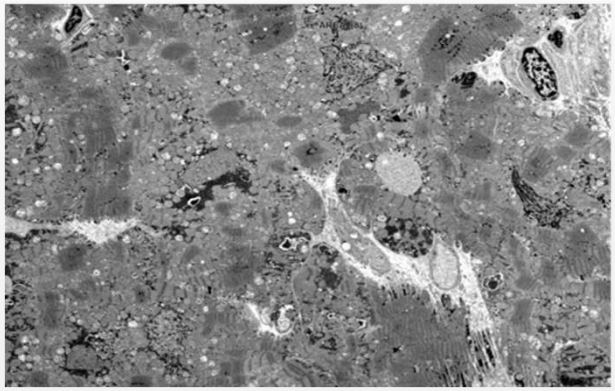
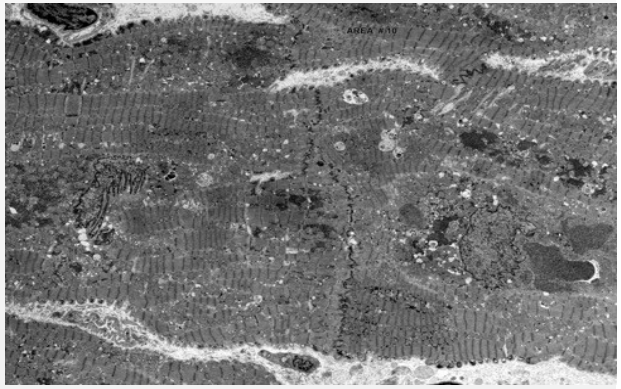
Robust LAMP2 Cardiac Protein Expression by Immunohistochemistry
Vacuole Reduction and **Restored Myofibrillar Structure** by Electron Microscopy

	Normal Control	Baseline*	Month 9*
Immunohistochemistry			
Electron Microscopy			

* Representative images from patient 1005: endomyocardial biopsy

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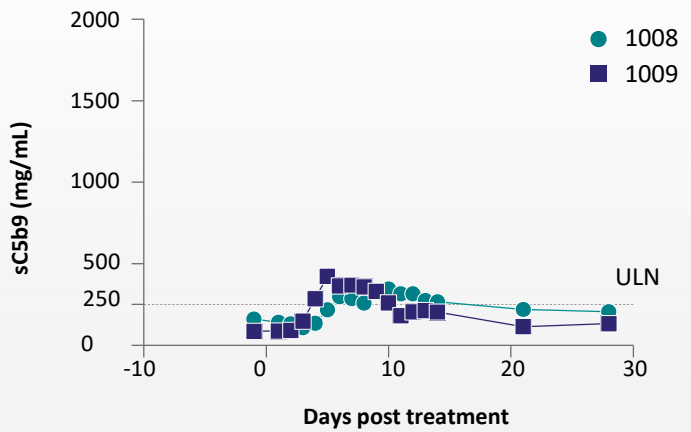
Manageable Safety Profile

Enhanced immunomodulatory regimen for pediatric cohort was associated with limited side effects: Mitigation of AEs observed in low- and high-dose adult cohorts

Limited complement activation
Low dose pediatric cohort
Rituximab + Corticosteroids + Sirolimus



Platelets remain in normal range for 2 of 2 pediatric patients

Rituximab + Corticosteroids + Sirolimus

- Minimal complement activation and ↓ potential for TMA
- Early steroid taper and no exacerbation of Danon disease-associated skeletal myopathy

Enhanced risk management plan: Safety results

- Infusion well tolerated with no drug-related SAEs
- Mitigation of complement activation as evidenced by normal-range platelets, hemoglobin and creatinine
- Baseline skeletal myopathy was not significantly exacerbated post treatment
- Patients have been clinically stable

AE, adverse event; SAE, serious adverse event; TMA, thrombotic microangiopathies; ULN, upper limit of normal.
Data on file. Rocket Pharmaceuticals. 2022.

Development Plan

Moving toward pivotal Phase 2 study

CURRENT

- Phase 1 treatment completed in males
- Orphan Drug, Rare Pediatric and Fast Track designations in the US (eligible for PRV)
- Initiated in-house manufacturing to support Phase 2 product

PLANNED

- Pediatric cohort update in late Q3 2022
- Expanded natural history study
- End of Phase 1 Regulatory meeting with FDA
- Initiate Phase 2 Global Pivotal Study Activities
- Initiate female study

PLANNED GLOBAL REGISTRATIONAL PHASE 2 STUDY

FDA, Food and Drug Administration; H2, second half of the year; PRV, priority review voucher.
Data on file. Rocket Pharmaceuticals. 2022.

Ex Vivo Platform: Lentiviral Vector (LV)

Fanconi Anemia, Leukocyte Adhesion Deficiency-I and Pyruvate Kinase Deficiency

- HSCs transduced with a lentiviral vector carrying the corrected gene and infused following transduction
- Transduction process occurs ex vivo, ensuring the gene has been properly integrated before the therapy is given to the patient
- Corrected HSCs engraft in bone marrow, and repopulate marrow and blood with functional hematopoietic cells capable of reversing disorder

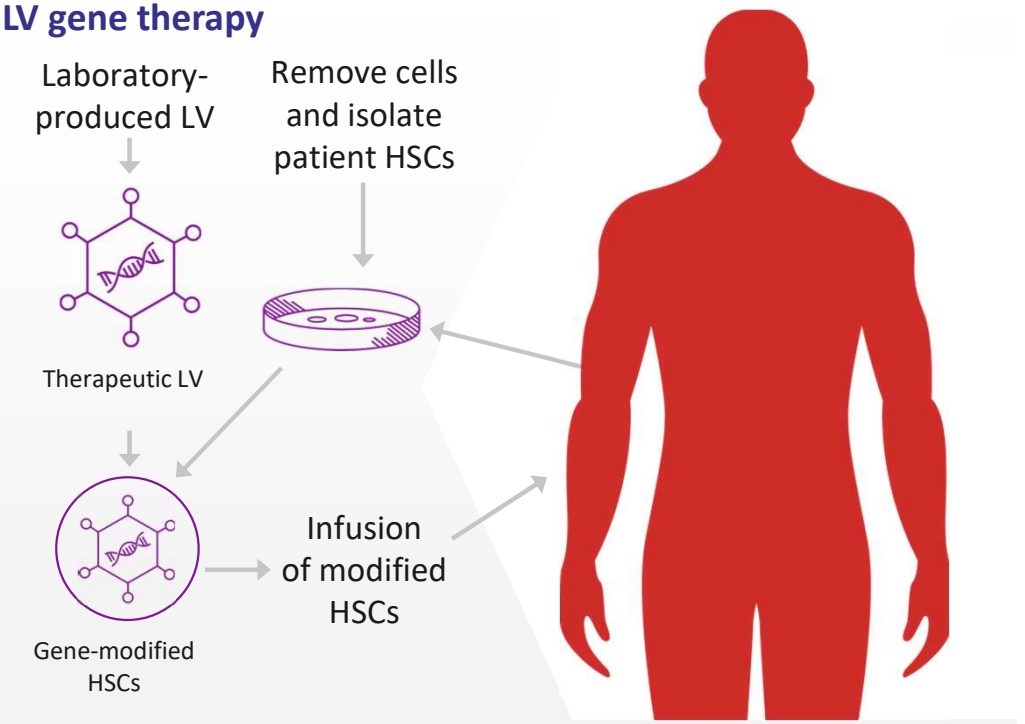
IDEAL FOR

Modifying HSCs to address hematologic and immune disorders

GOAL

Promote sufficient quantities of a healthy therapeutic protein to be manufactured by patients' own blood cells

EX VIVO (outside the body) LV gene therapy



HSC, hematopoietic stem cell; LV, lentiviral vector.
Data on file. Rocket Pharmaceuticals. 2022.

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

Fanconi Anemia (A, C, & 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 60-70% 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
  - Congenital abnormalities

BMF, bone marrow failure; FA, Fanconi Anemia; FA-A, Fanconi Anemia, group A; FANC, Fanconi Anemia complementation group; GvHD, graft-versus-host disease; HLA, human leukocyte antigen; HSCT, hematopoietic stem cell transplantation; MOA, mechanism of action.

Alter BP et al. *Br J Haematol.* 2010;150(2):179-188.

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Clinical Studies Overview

Description

Autologous HSCs transduced with LV carrying *FANCA* transgene
Conditioning is not required because gene-corrected HSCs display proliferative advantage over time

Clinical studies

- EU FANCOLEN I study (N=9) completed
- US Phase 1 study (N=2) completed
- US Phase 2 study ongoing
- EU Phase 2 study ongoing

Primary endpoints*:

- Engraftment (VCN)
- Phenotypic correction (BM MMC-resistance)
- Prevention of BMF (blood count stability)

Safety

- No conditioning
- No dysplasia, clonal dominance or oncogenic integrations
- 1 RP-L102 related SAE: infusion-related reaction (transient, Grade 2)

Key efficacy data

12 patients
Enrolled and treated

- **9 patients**
  With ≥12 months follow-up
  - **6 patients**
    Sustained peripheral blood and bone marrow genetic correction (VCN)
    → **5 patients**
    Sustained, increasing MMC resistance in BM CFCs:
    - 51% to 94% at 18 to 24 months, and
    - ≥20% at 2 consecutive timepoints
  - **2 patients**
    Potential for engraftment 12 to 24 months post RP-L102
  - **1 patient**
    Progressive BMF and underwent successful allogeneic transplant
- **3 patients**
  <12 months follow-up †

Evidence of confirmed phenotypic & genetic correction and hematologic stability identified in 5 of initial 9 evaluable patients

*Efficacy in ≥5 patients (observed over >1 year post prescription) required to reject null hypothesis. † In absence of conditioning, ≥12 months follow-up required to identify engraftment and MMC-resistance.
BM CFC, bone marrow colony forming cell; BMF, bone marrow failure; FA, Fanconi Anemia; HSC, hematopoietic stem cell; MMC, mitomycin-C; VCN, vector copy number.
Data on file. Rocket Pharmaceuticals. 2022.

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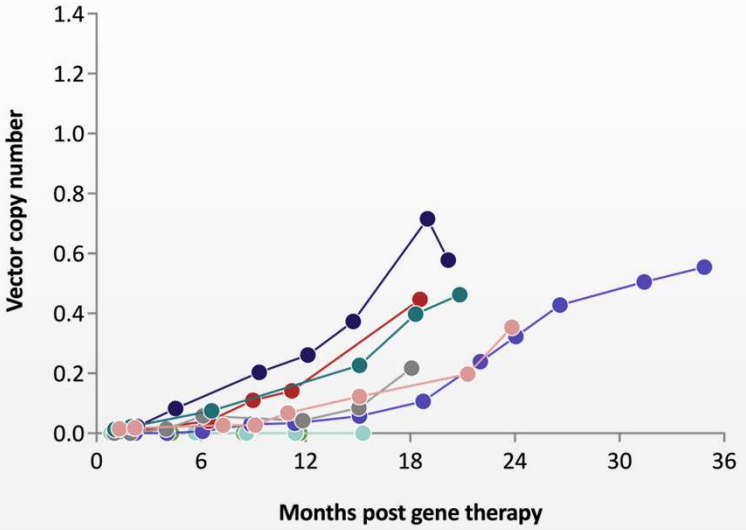
Progressive Increase in **Peripheral Blood and Bone Marrow VCNs**

Progressive increases in gene markings in peripheral blood and bone marrow cells in 6 patients

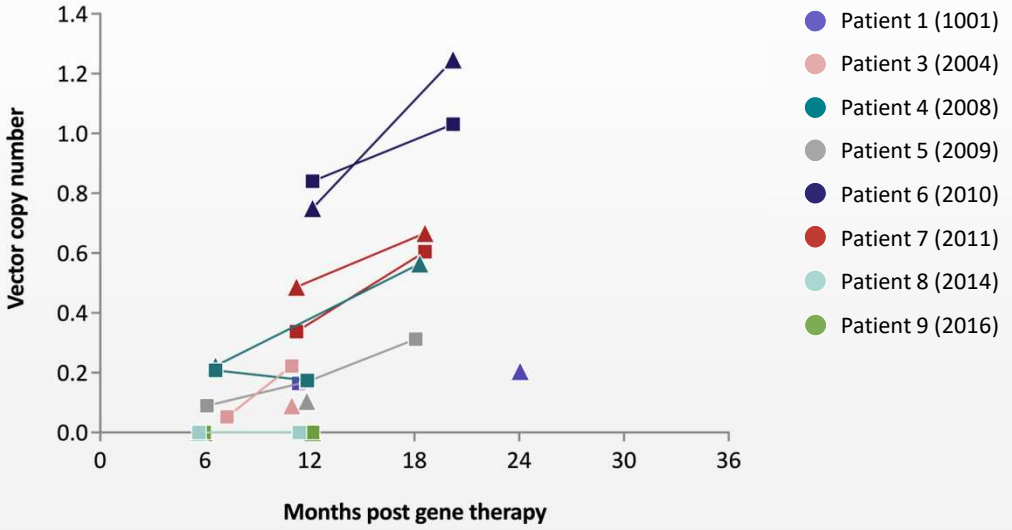
Peripheral blood VCN

○ VCN in peripheral blood mononuclear cells



Bone marrow VCN

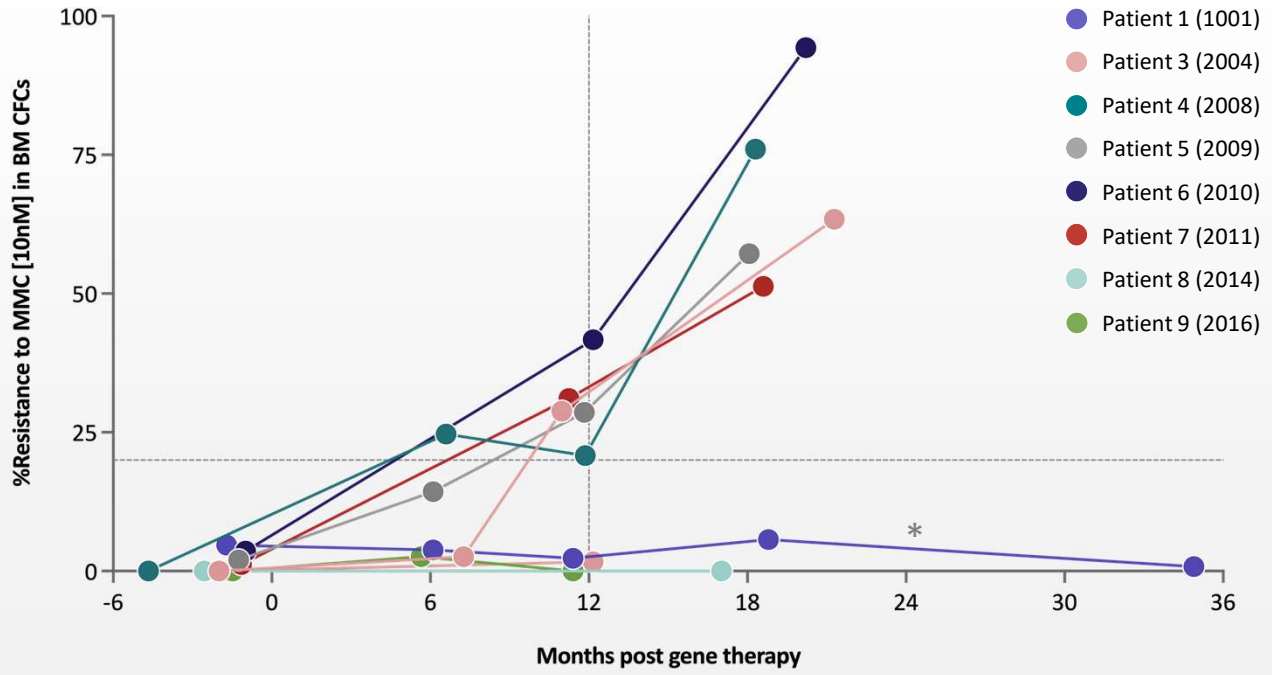
□ VCN in bone marrow mononuclear cells
△ VCN in bone marrow CD34+ cells



FA, Fanconi Anemia; VCN, vector copy number.
Data on file. Rocket Pharmaceuticals. 2022. Data Cut-off: April 4, 2022

Strong Evidence for **Phenotypic Reversal**

Increasing phenotypic correction over 1 to 2 years post RP-L102* in 5 of initial 9 evaluable patients

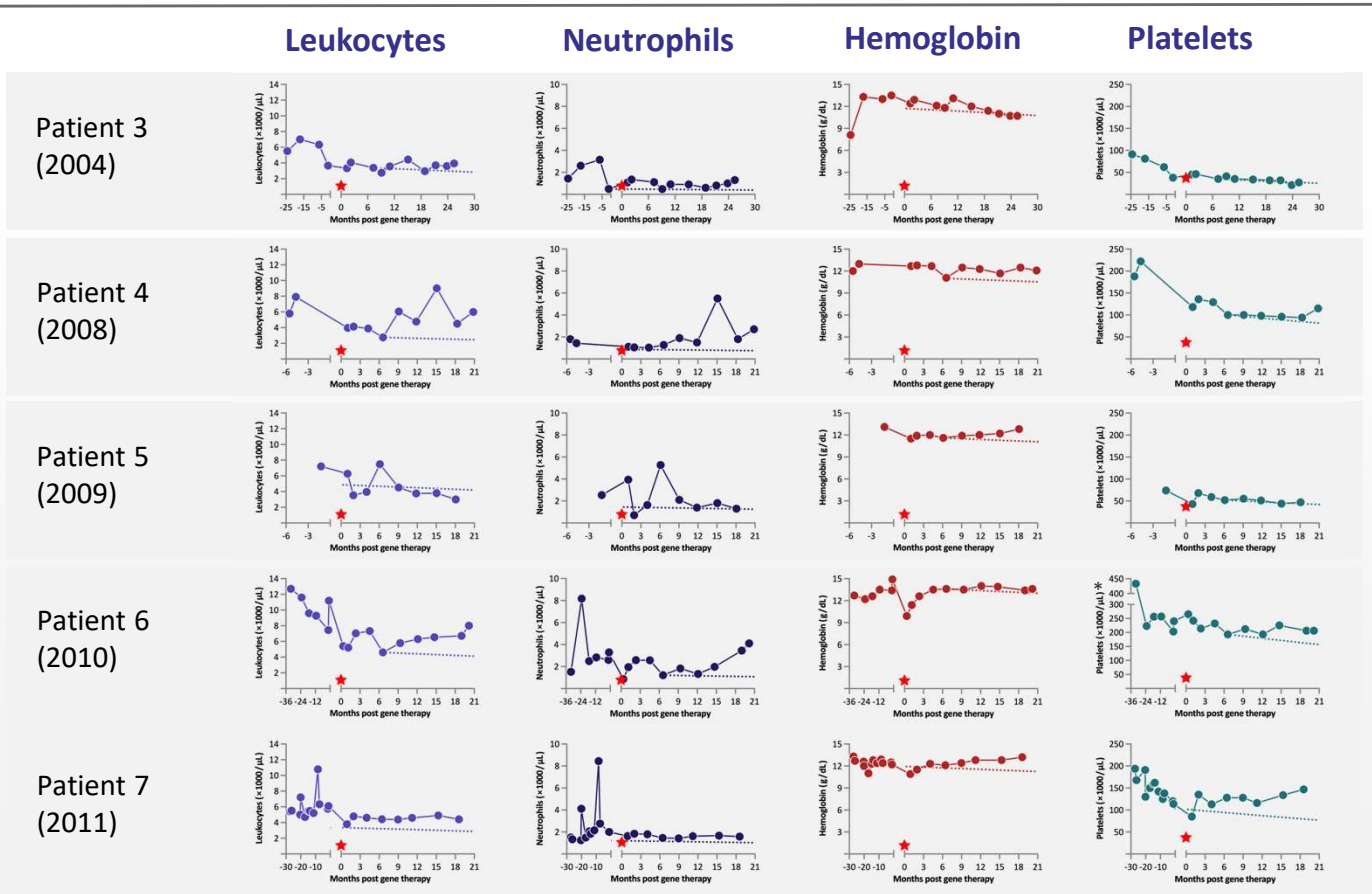


For 5 patients, increased BM CFC MMC resistance ranging from 51% to 94% observed at 18 to 24 months post–RP-L102 administration

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

*BM MMC-res for Patient 1 (1001)'s 24-month assessment was not performed at one of the study's central laboratories and is not included. Not shown: BM MMC-res in Patient 2 (1002), who was withdrawn from the study at 18 months post–RP-L102 infusion.
BM CFC, bone marrow colony forming cell; FA, Fanconi Anemia; MMC, mitomycin-C; VCN, vector copy number.
Data on file. Rocket Pharmaceuticals. 2022. Data Cut-off: April 4, 2022

Blood Count Stabilization and Sustained Phenotypic Reversal



Increased MMC resistance in BM CFCs associated with hematologic stabilization at ≥1 year post RP-L102

Concomitant blood count stabilization over 12 to 24 months seen in all 5 patients with sustained and increasing BM CFC MMC resistance

☆ Time of RP-L102 Infusion

····· Projected blood counts based on FA-A natural history

BM CFC, bone marrow colony forming cell; FA, Fanconi Anemia; MMC, mitomycin-C.
Data on file. Rocket Pharmaceuticals. 2022. Data Cut-off: April 4, 2022

* Y-axis for Pt 2010 platelet counts is different scale to incorporate pre-treatment values

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Development Plan

Moving toward BLA/MAA filing

INITIAL EFFICACY AND HIGHLY FAVORABLE SAFETY PROFILE

- Initial comprehensive efficacy in 5/9 evaluable patients (≥12-month f/u)
- No cytotoxic conditioning, only 1 transient RP-L102 related SAE (Grade 2)

REGULATORY DESIGNATIONS

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

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

ANTICIPATED SIMULTANEOUS BLA/MAA FILING

Additional life-cycle management activities:

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

ATMP, advanced therapy medicinal product; BLA, Biologics License Application; FA, Fanconi Anemia; 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. 2022.

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

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 HSC transplant
- **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 y in absence of alloHSCT
  - Moderate LAD-I: Death prior to age 40 in >50% of patients, extensive morbidity with recurrent infections and inflammatory lesions

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

GvHD, graft-versus-host disease; HSC, hematopoietic stem cell; ITGB2, integrin subunit beta 2; LAD-I, Leukocyte Adhesion Deficiency-I; MOA, mechanism of action.

Almarza E et al. *J Allergy Clin Immunol Pract.* 2018;6(4):1418-1420.

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Clinical Study Overview

Description

Autologous HSCs transduced with LV carrying *ITGB2* transgene

Clinical study

Treatment completed

Phase 1/2 (N=9)

Primary endpoints:

- Safety (Phase 1)
- Survival and safety (Phase 2)

Selected secondary endpoints:

- CD18 expression
- Genetic correction
- Incidence of infections
- Overall survival

Safety

- Well tolerated; no drug product-related SAEs
- No graft rejection, no GvHD
- Initial ISA indicates highly polyclonal patterns without evidence of dominant integrations in proximity to oncogenic loci

Key efficacy data

- 100% overall survival
- Efficacy evident in 9/9 patients – genetic, laboratory and clinical reversal of disease course
- Sustained ≥ 10% CD18 neutrophil expression, concomitant sustained CD11 expression, VCN of ≥ 0.1 in PB neutrophils and leukocytosis resolution
- Significant reduction in all hospitalizations, including infection- and inflammatory-related hospitalizations, prolonged hospitalizations and severe infections
- Spontaneous resolution of LAD-I–related skin rash and restoration of wound repair capabilities

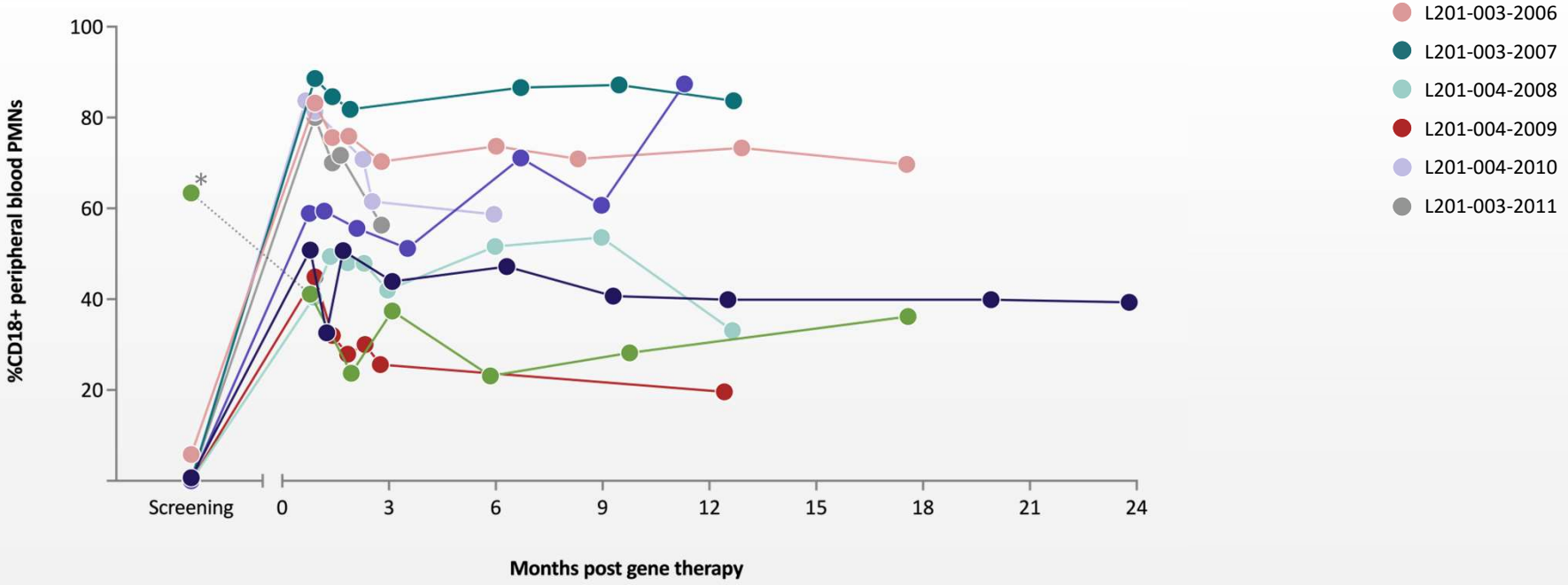
GvHD, graft-versus-host disease; HSC, hematopoietic stem cell; ISA, Insertion site analysis; LAD-I, Leukocyte Adhesion Deficiency-I; PMN, polymorphonuclear neutrophils; SAE, serious adverse event; VCN, vector copy number.

ClinicalTrials.gov. NCT03812263. Accessed May 9, 2022. https://clinicaltrials.gov/ct2/show/NCT03812263

Data Cut-Off March 9, 2022

Sustained **CD18 Expression in Peripheral Blood PMNs**

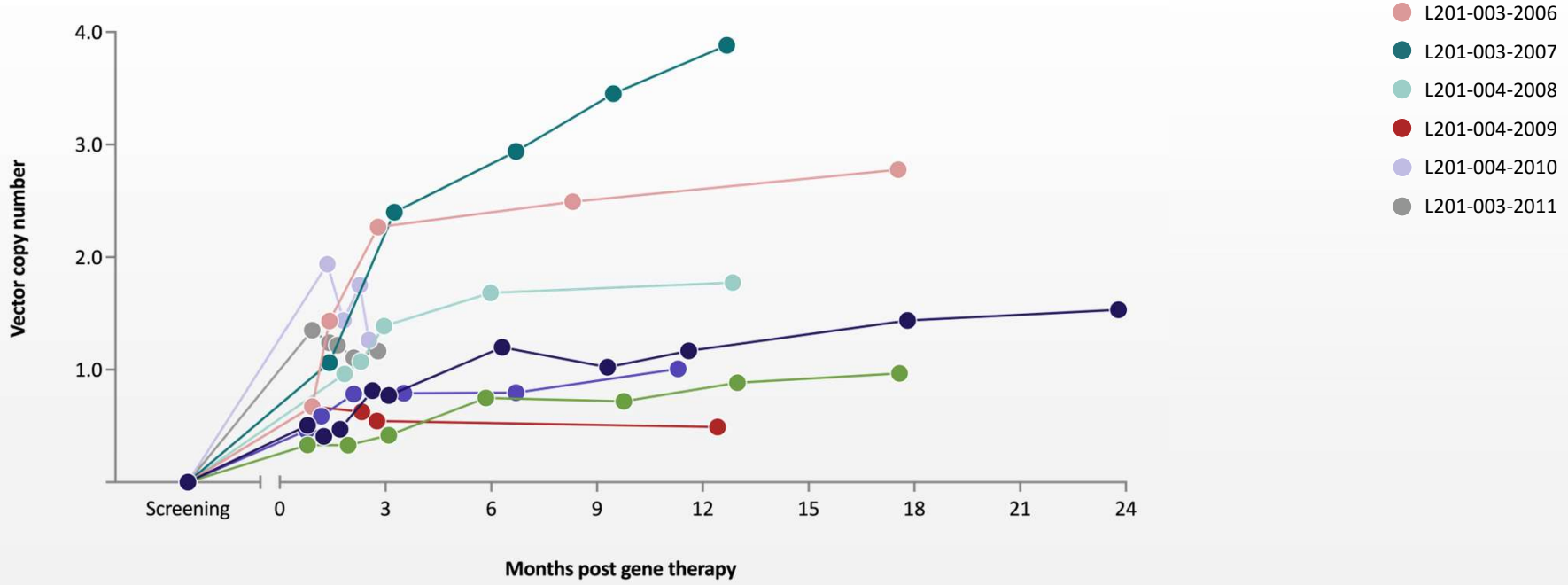
At 3 to 24 months after infusion, 9/9 patients sustained stable CD18 expression (median: 56%) with no therapy-related serious adverse events



*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: March 9, 2022

Sustained **VCN in PBMCs**

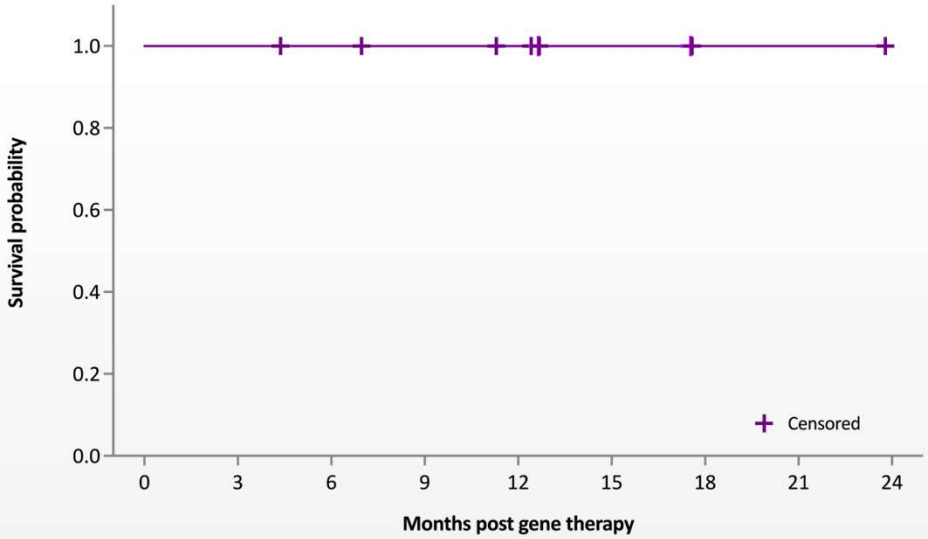
At 3 to 24 months after infusion, 9/9 patients sustained stable CD18 expression (median: 56%) with no therapy-related serious adverse events



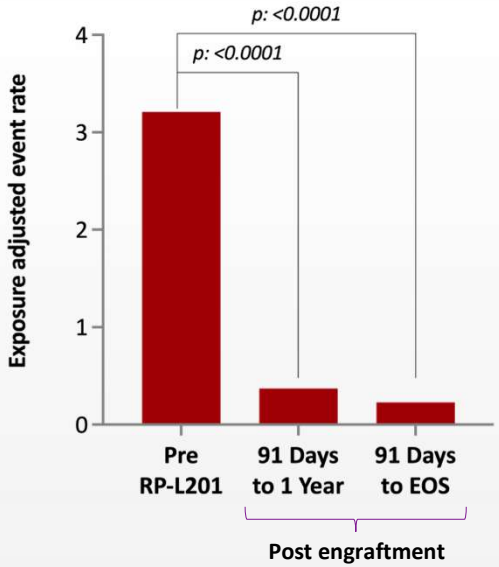
LAD-I, Leukocyte Adhesion Deficiency-I; PB, peripheral blood; PBMCs, peripheral blood mononuclear cells; VCN, vector copy number.
Data on file. Rocket Pharmaceuticals. 2022. Data Cut-Off: March 9, 2022

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

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

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Development Plan

Moving toward BLA/MAA 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

REGULATORY DESIGNATIONS

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

TOP-LINE DATA READOUT 2Q 2022

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

Guiding H1 2023 **BLA/MAA Filing**

Life-cycle management

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

ATMP, advanced therapy medicinal product; BLA, Biologics License Application; H1, first half of the year; LAD-I, leukocyte adhesion deficiency-I; MAA, Market Authorization Application; PRIME, PRIority MEdicines; PRV, priority review voucher; RMAT, Regenerative Medicine Advanced Therapy.
Data on file. Rocket Pharmaceuticals. 2022. Data Cut-off: March 9, 2022

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

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

Market Opportunity – US and EU

Prevalence of **4,000 to 8,000** individuals

Annual Incidence of **75 to 125** individuals

LV, lentiviral vector; MOA, mechanism of action; PKD, pyruvate kinase deficiency; PKLR, pyruvate kinase L/R; RBC, red blood cell.

Zanella A et al. *Br J Haematol.* 2005;130(1):11-25.

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Clinical Study Overview

Description

Autologous HSCs transduced with LV containing human *PKLR* transgene

Clinical study

N = 4-5 (Phase 1)

Primary endpoints:

- Safety
- Toxicity

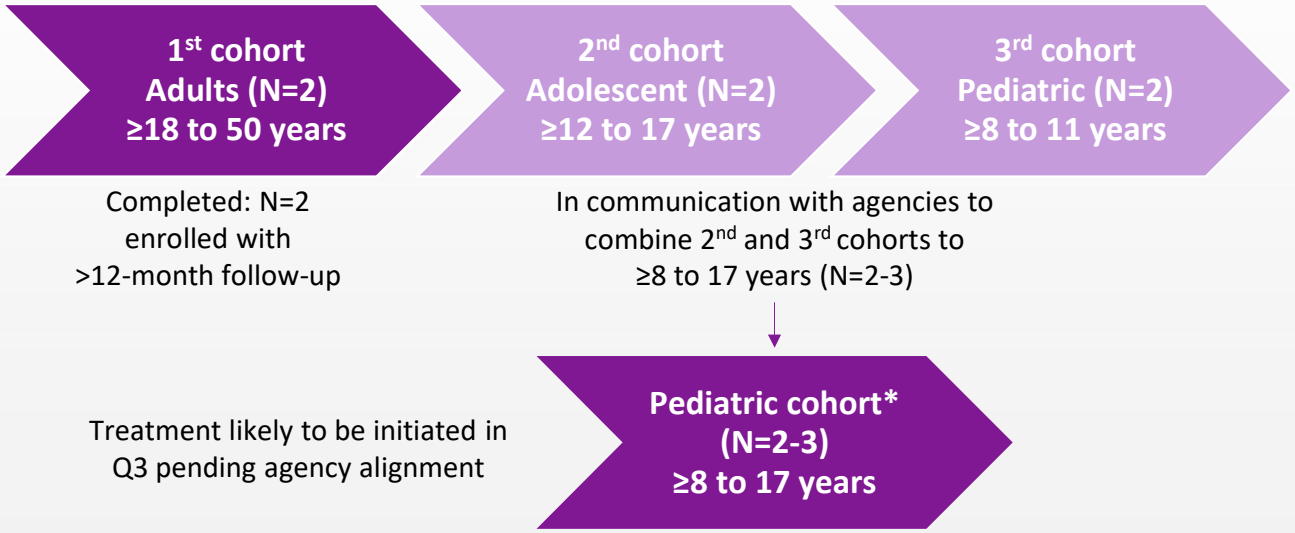
Selected secondary endpoints:

- Genetic correction
- Transfusion independence
- Reduction in anemia
- Reduction of hemolysis

Safety

Appears favorable with no IP-related SAEs

Phase 1 cohort dosing plan



*Discussions ongoing w/ agency.

HSC, hematopoietic stem cell; IP, investigational product; LV, lentiviral vector; PKD, Pyruvate Kinase Deficiency; Q2, second quarter of the year; SAE, serious adverse event.

ClinicalTrials.gov. NCT04105166. Accessed May 9, 2022. https://clinicaltrials.gov/ct2/show/NCT04105166

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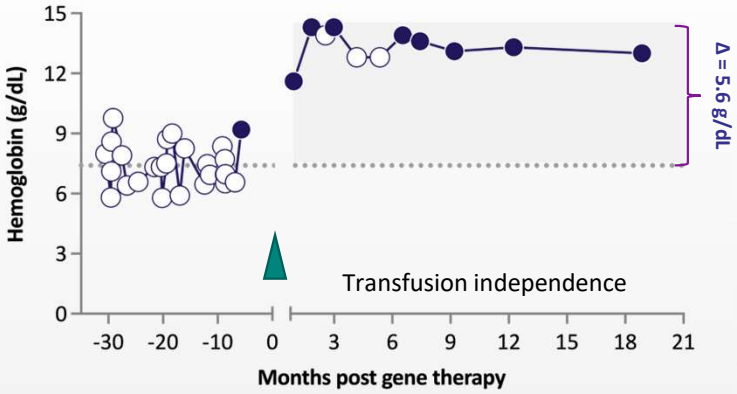
Hemoglobin Normalization and Transfusion Independence

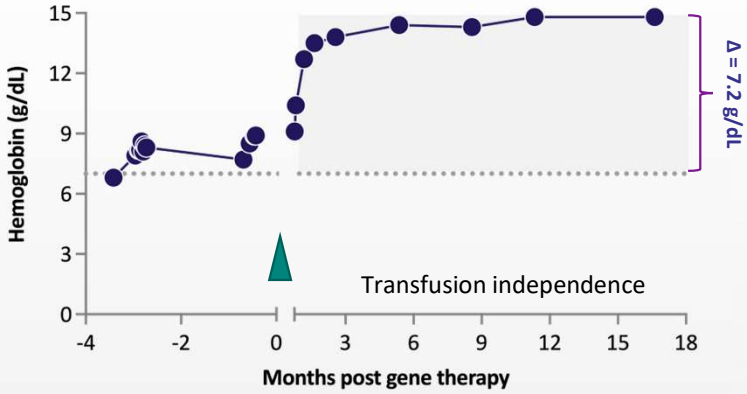
Hemoglobin improvement to normal range (from baselines in severe (<8g/dL range)
Transfusion independence (extensive transfusion requirements prior to RP-L301)
Sustained improvement of hemolysis markers (LDH, bilirubin) and PB VCNs in 1.0 – 3.0 range

PATIENT 1001



- Hemoglobin normalized (from ~7.4 to 13.0 g/dL) sustained at 18 months post infusion
- No transfusion requirements following engraftment

PATIENT 1002



- Hemoglobin normalized (from ~7.0 to 14.8 g/dL) sustained at ~18 months post infusion
- No transfusion requirements following engraftment
- Prior therapy with mitapivat: no Hb significant increase

Dotted lines indicate average Hb for each patient prior to gene therapy

PKD, Pyruvate Kinase Deficiency; pVCN, peripheral vector copy number.
Data on file. Rocket Pharmaceuticals. 2022. Data Cut-off: April 13, 2022

Time of RP-L301 infusion

Development Plan

Moving toward pivotal Phase 2 study

PKD STUDY PROGRESS TO PHASE 2 AND LAUNCH

Key endpoints selected

- Hemoglobin increase
- ↓ 50% transfusions or transfusion independence

Well-delineated natural history in recent PKD NHS publications

- Complete Phase 1 pediatric cohort dosing (N=2-3)
- End of Phase 1 regulatory meeting with FDA
- 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)

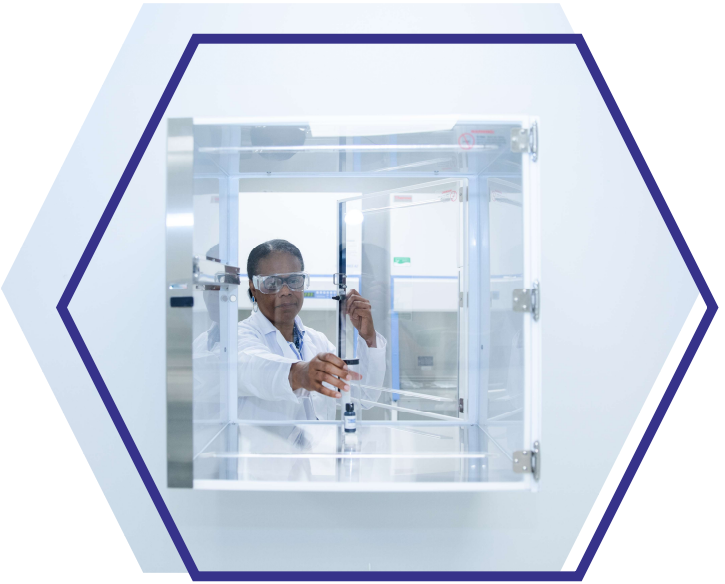
LIFE-CYCLE MANAGEMENT

ANTICIPATED EXPANSION STUDY TO PRE-SPLENECTOMY PATIENTS IN 2023

EXPLORATION OF NON-GENOTOXIC CONDITIONING

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

FUTURE DIRECTIONS



FUTURE DIRECTIONS

Rocket Pharmaceuticals: **Elevating Gene Therapy to New Heights**

- Recognized as a premier gene therapy company
- Specialized against monogenic diseases
- Pioneer in the development of both *ex vivo* LV and *in vivo* AAV therapies
- AAV9-based gene therapy for Danon disease, a major value driver based on size of indication and lack of other therapies
- LV-based programs to provide near term commercialization

- Commercial company with initial therapies and revenue build for Danon disease, FA, LAD-I and PKD
- Broad pipeline of additional new therapies targeting potentially larger opportunities for rare and orphan diseases
- Potential new technologies employed (gene editing and non-viral gene therapies)

AAV, adeno-associated virus; FA, Fanconi Anemia; LAD-I, Leukocyte Adhesion Deficiency-I; LV, lentiviral vector; PKD, Pyruvate Kinase Deficiency.
Data on file. Rocket Pharmaceuticals. 2022.

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FUTURE DIRECTIONS

Anticipated Milestones and **Wave 2**

2022

Q2 2022
- LAD-I: Top-line data will be achieved by June
- FA: Top-line data achieved early
- In-house AAV cGMP manufacturing

Q3 2022
- Danon disease: Pediatric cohort data

Q4 2022
- Danon disease: Initiate Phase 2 pivotal study activities
- FA, PKD: Additional data

2023
- BLA/MAA filing for LAD-I
- BLA/MAA filing for FA
- PKD: Initiate Phase 2 pivotal study activities
- Wave 2 pipeline enters clinic

2024
- Label expansion studies for Danon disease (female), FA (C & G), LAD-I (moderate) and PKD (moderate) programs

AAV, adeno-associated virus; BLA, Biologics License Application; cGMP, current Good Manufacturing Processes; FA, Fanconi anemia; H1, first half of the year; LAD-I, leukocyte adhesion deficiency-I; PKD, pyruvate kinase deficiency; Q2, second quarter of the year; Q3, third quarter of the year; Q4, fourth quarter of the year.
Data on file. Rocket Pharmaceuticals. 2022.

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FUTURE DIRECTIONS

Future Therapies: Wave 2 (AAV)

Focused R&D Strategy for Sustainable Innovation

First-, best- and only-in-class

On-target MOA; clear endpoints

Sizeable market to maximize patient impact

We continue to build our pipeline based on our core R&D strategy; identifying the "most productive" indications for the most efficient development path.

MOA, mechanism of action; R&D, research & development
Data on file. Rocket Pharmaceuticals. 2022.

THANK YOU!

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