
rocket
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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.

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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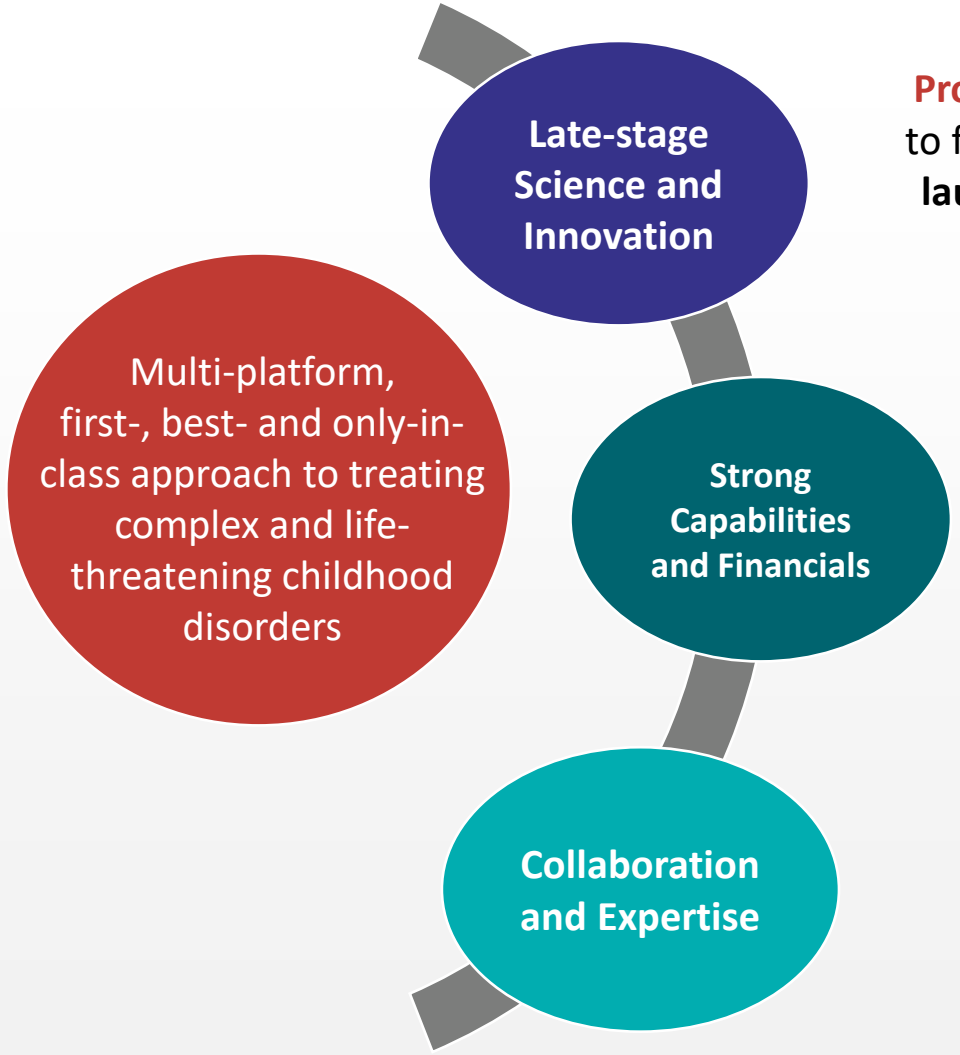
Vision: Seeking Gene Therapy Cures

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

$306.5M
in cash and cash equivalents; with additional net proceeds of
$108.2M
from recent equity offering

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

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

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

Pfizer · AstraZeneca · Roche · Bristol Myers Squibb · NOVARTIS

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

NOVARTIS · Andelyn Biosciences · Celgene · MERCK

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

teva · Shire · ...ichnos...

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

NOVARTIS · Advanced Accelerator Applications, A Novartis Company · Lilly · MERCK

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

Celsion Corporation · Osiris · caladrius BIOSCIENCES · PCT

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

FDA U.S. FOOD & DRUG ADMINISTRATION · SIDLEY

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

NewYork-Presbyterian Weill Cornell Medical Center · Mount Sinai · ImClone Systems · Lilly

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

endo · PAR Pharmaceutical, an endo international company · ...ichnos... · Teligent

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

LEGEND BIOTECH · J.P.Morgan · Bank of America Merrill Lynch

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

J&J · Bristol Myers Squibb · FDA U.S. FOOD & DRUG ADMINISTRATION · endo

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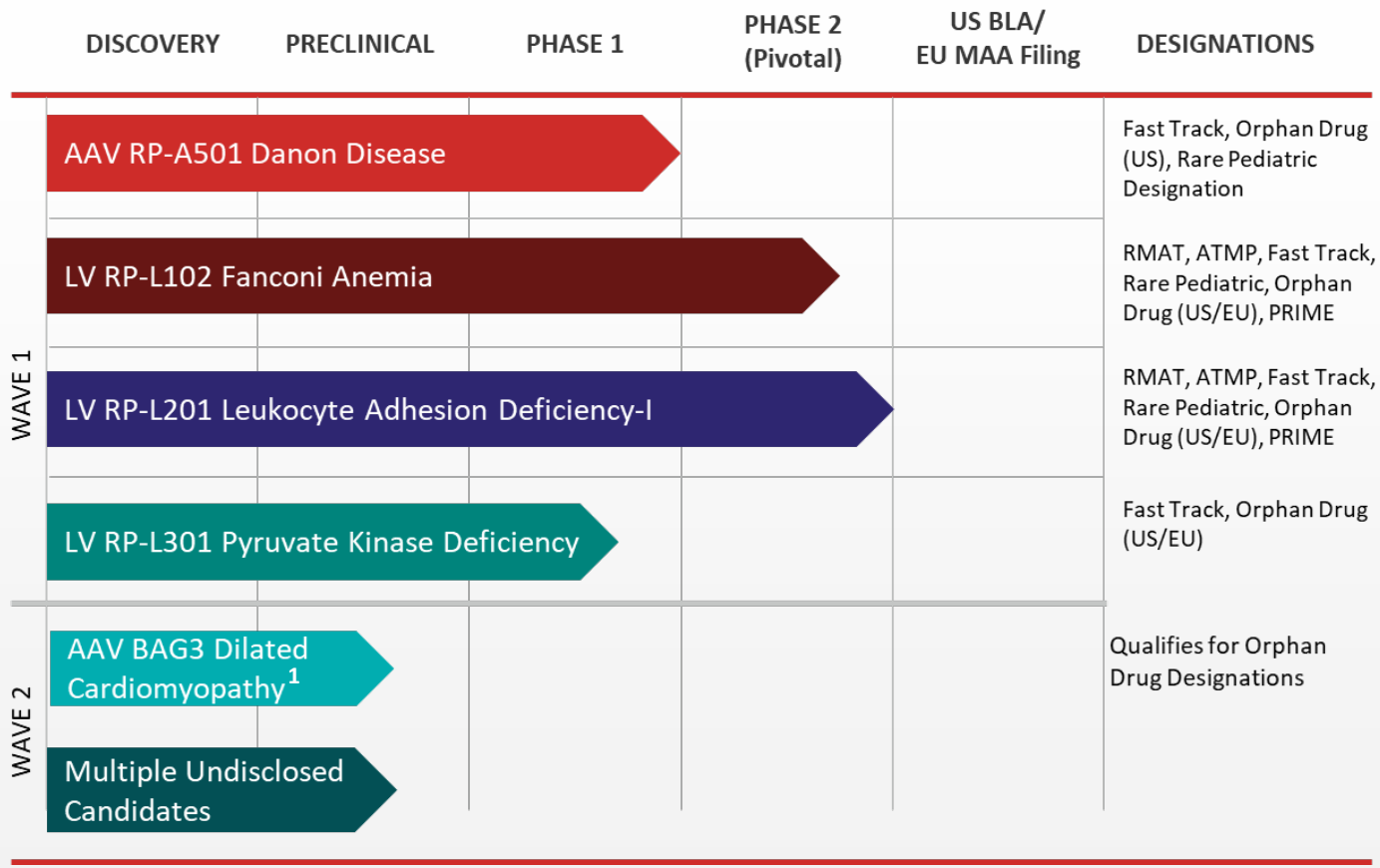
Strong Science, Carefully-selected Assets and Smart Execution:
Four Programs With Compelling Clinical POC

Criteria used to select programs

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

Five programs with compelling clinical proof of concept



[1]**Pending planned acquisition of Renovacor; transaction currently expected to close by Q1 2023.**

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

- ✓ **FA**: Guidance on BLA filing timeline
- **Danon**: FDA EOP1 agreement and initiation of Phase 2 pivotal study activities
- **PKD**: Phase 1 adult cohort data

2023

- **LAD-I**: BLA/MAA filings in 1H'23
- **FA**: BLA/MAA filings in 2H'23
- **PKD**: Phase 1 update in 1H'23; initiation of Phase 2 pivotal study in 2023
- Wave 2 **(including BAG3-DCM)** pipeline enters clinic[1]

[1]**Pending planned acquisition of Renovacor; transaction currently expected to close by Q1 2023.**

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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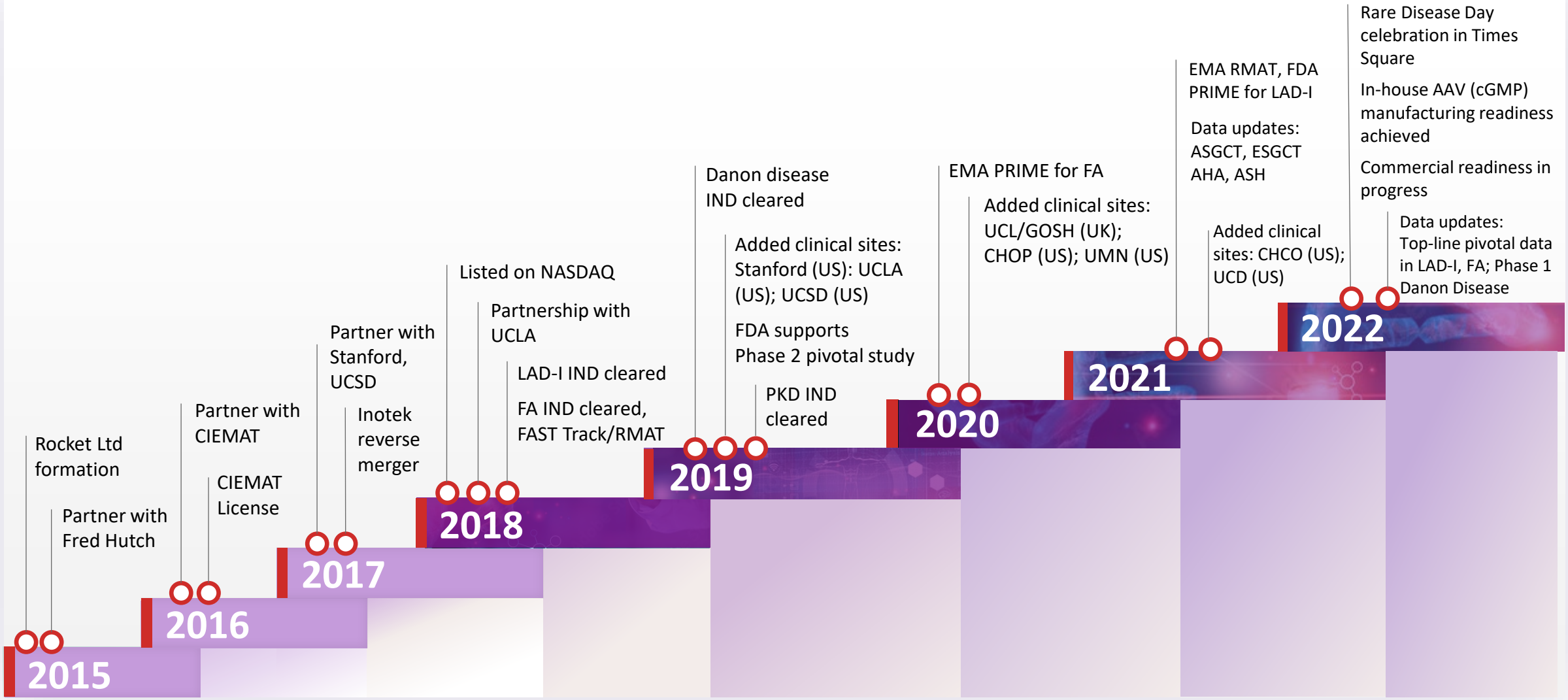
Strategically Building a Leading Gene Therapy Company



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.

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 David Geffen School of Medicine

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 Boulder | Colorado Springs | Denver | Anschutz Medical Campus

Penn

Stanford Medicine

Hospital Infantil Universitario Niño Jesús

UC San Diego

University of Minnesota

Hospital Universitario Fundación Jiménez Díaz Grupo quirónsalud

CIRM California's Stem Cell Agency

ciber Centro de Investigación Biomédica en Red

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

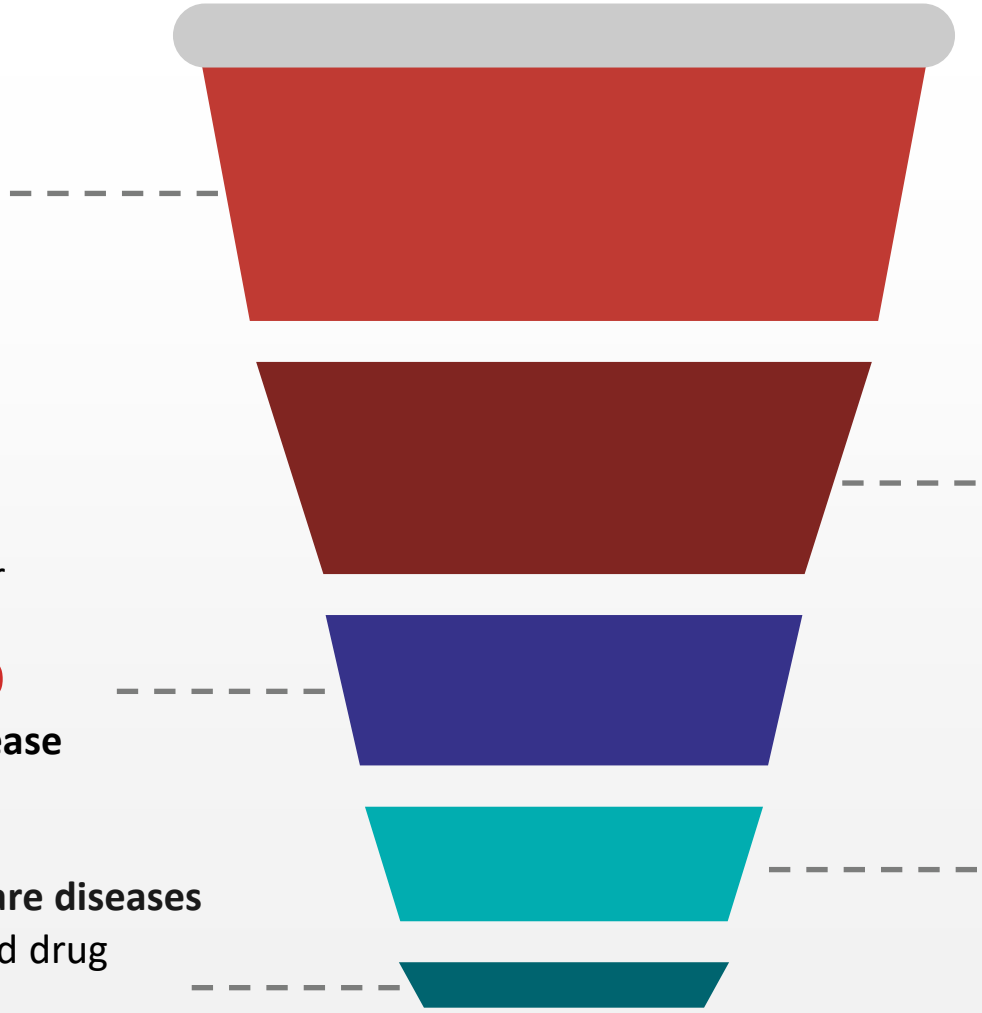
people globally are affected by a rare disease[1]

Children account for

50%

of rare disease patients[1]

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



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

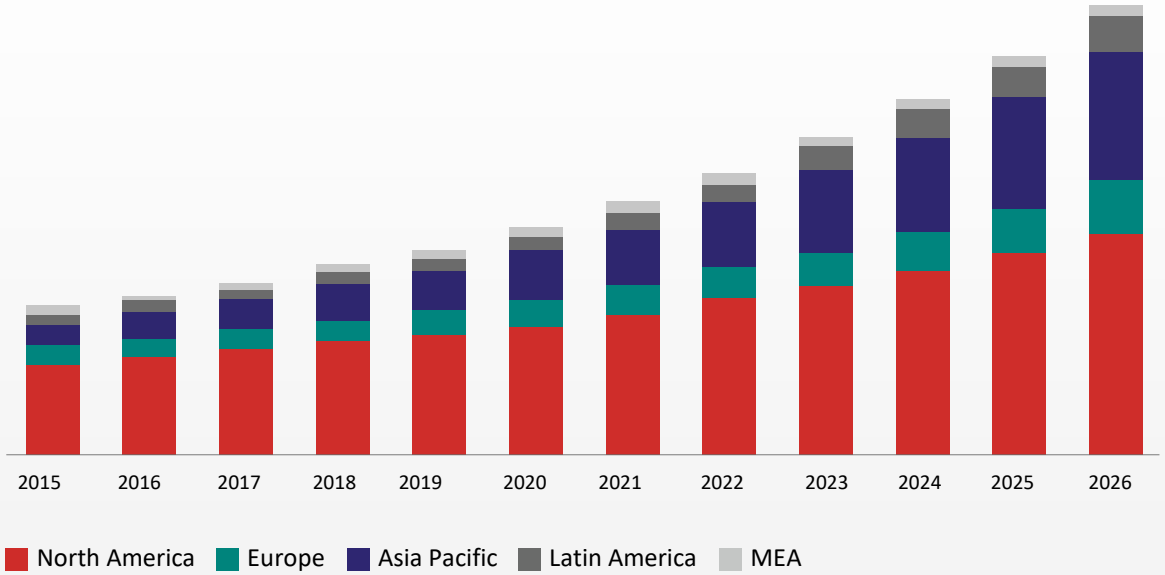
3 of 10 children with a rare disease die before their fifth birthday[1]

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]

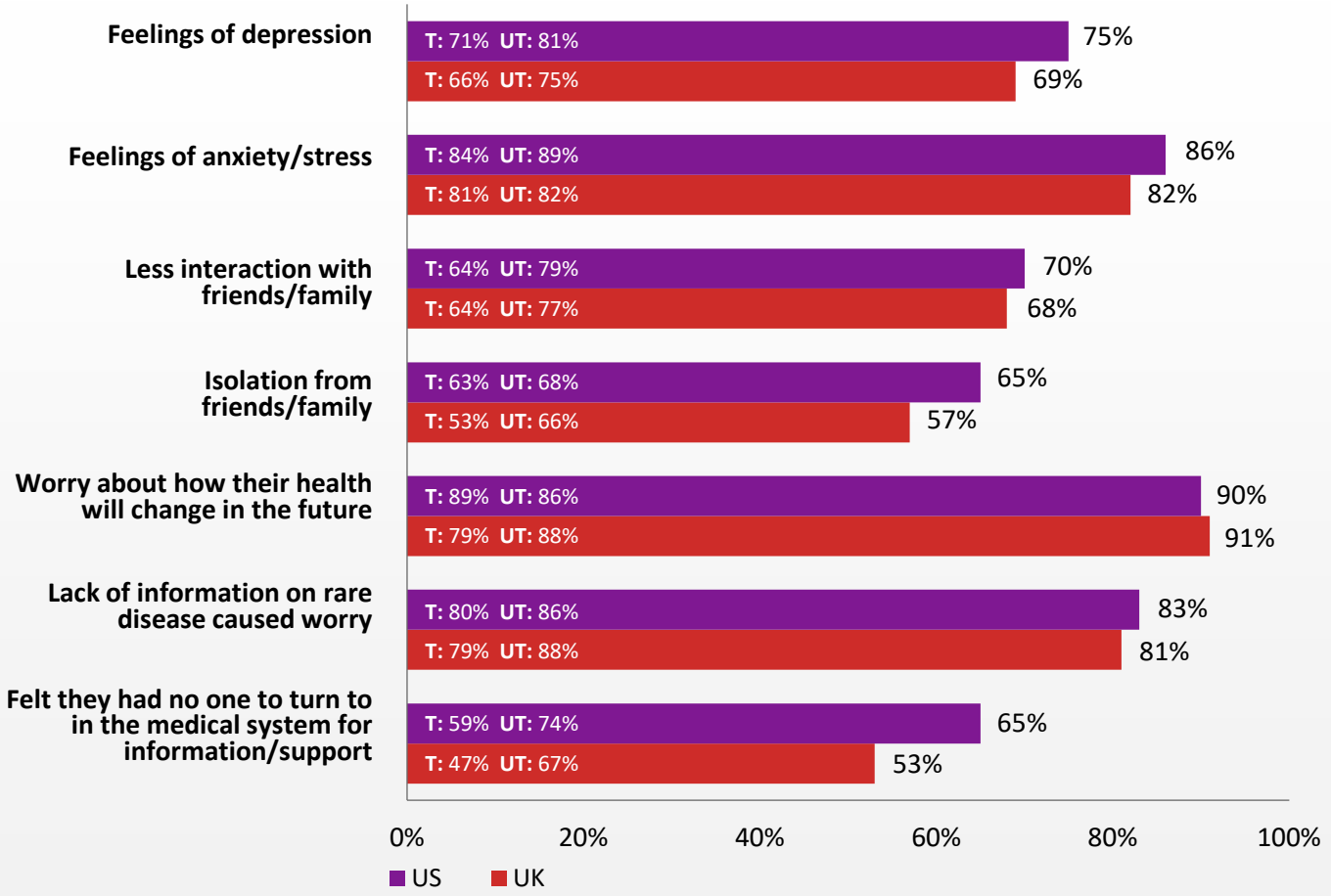
Economic impact[1]

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

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**

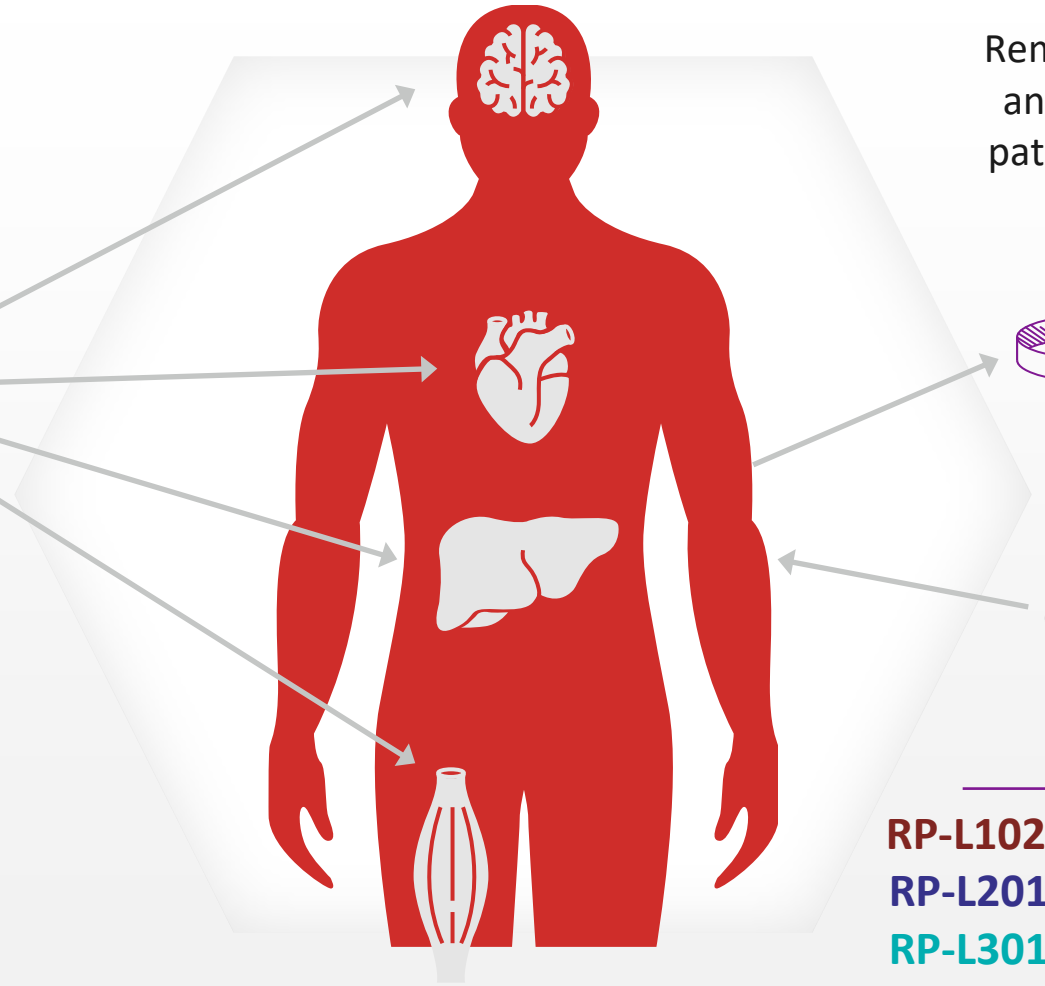
IN VIVO platform

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

Gene-modified HSCs

Infusion of 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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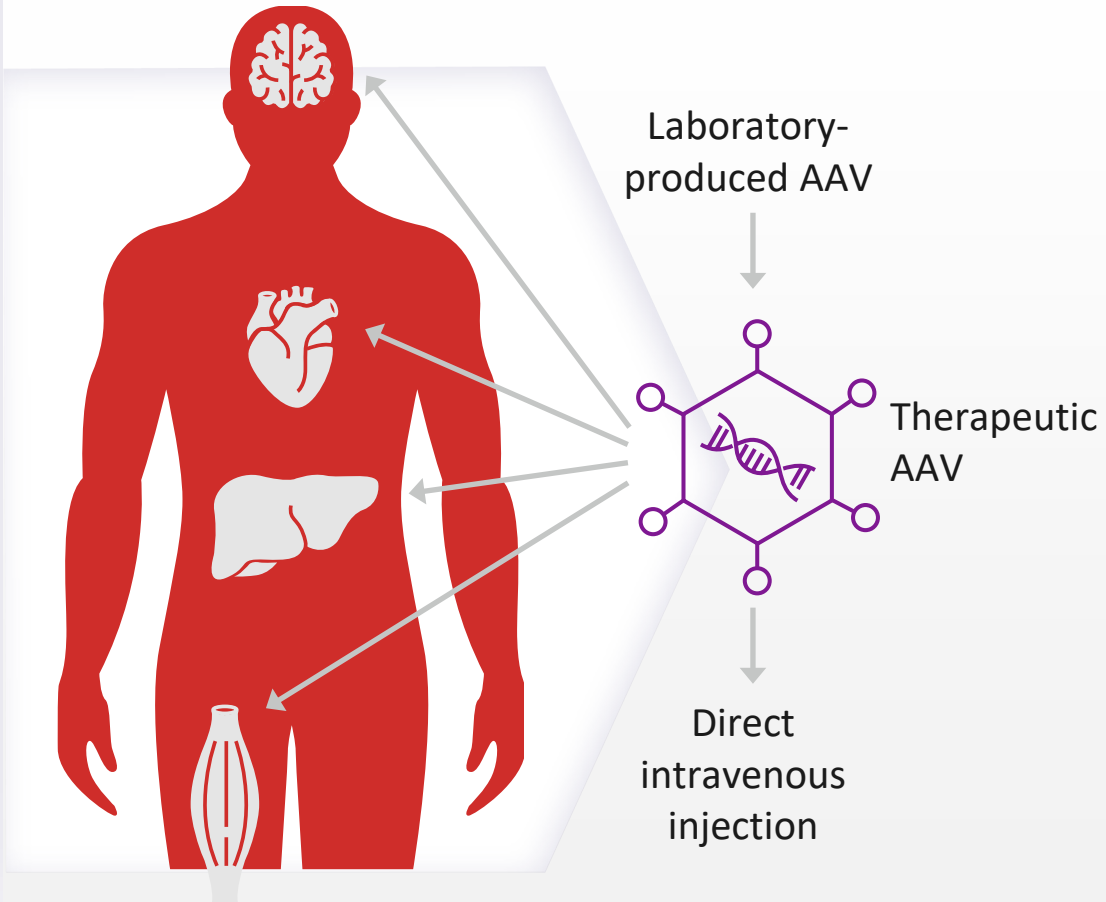
In Vivo Platform: Adeno-associated Virus (AAV)

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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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 *LAMP-2B*

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 et al. *Genet Med.* 2011;13(6):563-568. Brambatti M et al. *Int J Cardiol.* 2019;286:92-98.

RP-A501: Prospect of Direct Benefit

Trajectory of cardiac pathophysiology and heart failure in male Danon disease patients

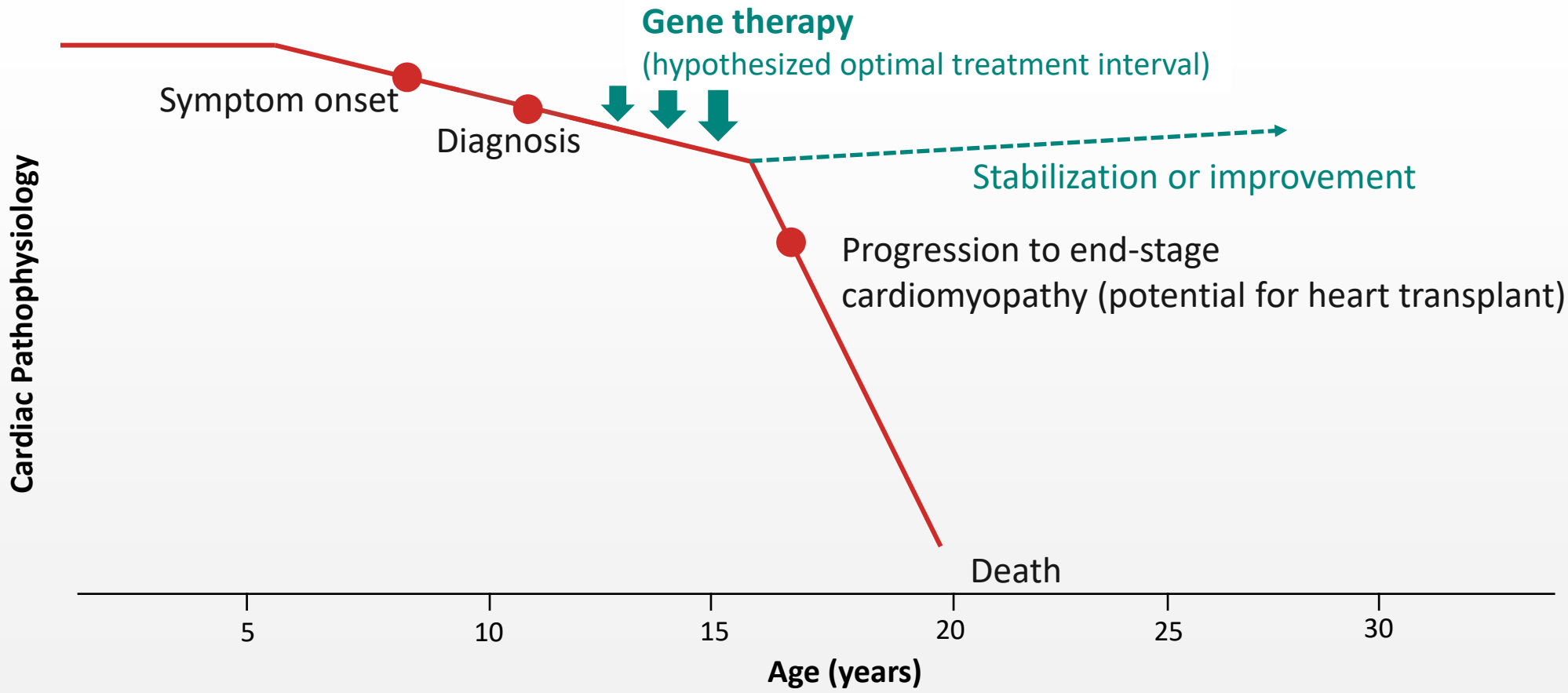
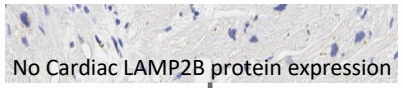


Figure modified from disease progression by age data from Boucek D et al. *Genet Med.* 2011;13(6):563-568 and Brambatti M et al. *Int J Cardiol.* 2019;286:92-98.

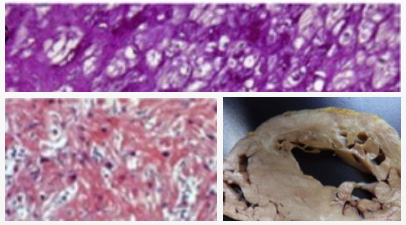
Pathophysiology and Clinical Manifestations of Danon Disease HCM

Pathophysiology

Pathogenic *LAMP-2B* mutation causing DD

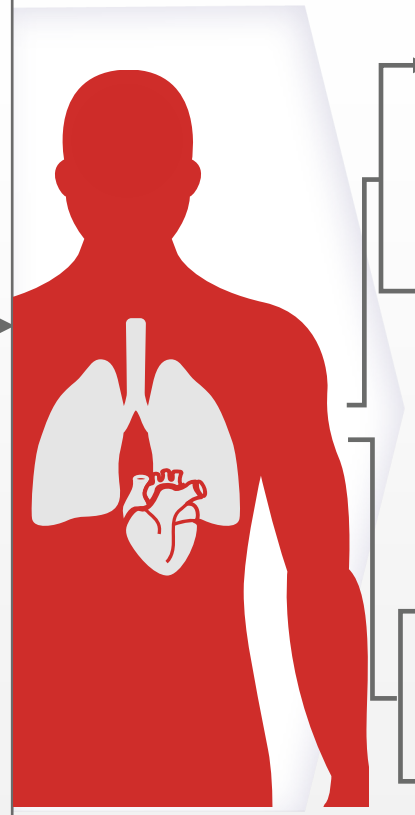


Autophagic vacuoles, myocardial disarray, myocardial thickening



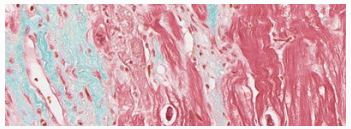
Impaired ventricular filling during diastole (diastolic dysfunction)

Mechanism



Disorganized myocytes disrupt signal conduction → **Ventricular arrhythmias** → **Sudden cardiac arrest**

Microvascular ischemia, apoptosis with fibrous substitution → Fibrosis



Fibrosis → Impaired systolic function → **End-Stage Heart Failure**

Decreased cardiac output

↓ Coronary artery perfusion, mismatch of myocardial oxygen supply & demand → Myocardial injury

Tissue hypoxia → Stimulus to breathe

Decreased cardiac output → **Fatigue**

Stimulus to breathe → **Tachypnea**

↑ Left-sided pressure → Pulmonary edema and ↑ work of breathing → **Dyspnea on exertion**

Patient impact

DD, Danon disease; LAMP2B, lysosome-associated membrane protein 2B; VT, ventricular tachycardia.
Adapted from Jones & Arnold. 2014. Hypertrophic Cardiomyopathy: Pathogenesis and Clinical Findings

Phase 1 Study: Non-Randomized Open Label

Non-randomized open label study in male DD patients

INCLUSION CRITERIA

- Male
- Confirmed *LAMP-2B* mutation
- Cardiac involvement confirmed by imaging or ECG
- NYHA Class II or III
- Able to walk >150 m unassisted during 6-minute walk test (6MWT)

EXCLUSION CRITERIA

- Anti-AAV9 neutralizing antibody titer >1:40
- Cardiopulmonary instability
- Prior organ transplantation
- LVEF <40% (implemented prior to pediatric cohort)

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

Pediatric
8-14 years
n=2 at CHOP

Single Intravenous Dose of RP-A501 (AAV9.LAMP2B)

Low Dose:
6.7×10^{13} GC/kg

High Dose*:
1.1×10^{14} GC/kg
*No further enrollment at this dose.

Enrollment Complete

36 months

PRIMARY OUTCOMES

- **Early and long-term safety**
- Target tissue transduction & 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
- Data cut September 27, 2022; source data verification through July 11, 2022.

6MWT, 6-minute walk test; AAV, adeno-associated virus; CHOP, Children's Hospital of Philadelphia; DD, Danon disease; ECG, electrocardiogram; *LAMP-2B*, lysosome-associated membrane protein 2B; LVEF, left ventricle ejection fraction; NYHA, New York Heart Association; UCSD, University of California San Diego.

RP-A501: Safety Monitoring of Phase 1 Patients

Cohort 1: Low-dose adult

Pt	Age	Wt
1001[a]	17.5	52.5
1002	20.4	89.1
1005	18.3	91.8

RP-A501 6.7 x 10^{13} GC/kg

Steroid (prednisone 1mg/kg)

Tacrolimus

SAEs of Interest
n=2 grade 3 steroid myopathy

Platelets
n=2, Grade 1 decrease (to LLN range)

Cohort 2: High-dose adult

Pt	Age	Wt
1006	21.1	82.7
1007[b]	20.7	96.7

RP-A501 1.1 x 10^{14} GC/kg

Steroid (prednisone 1mg/kg)

Tacrolimus

Rituximab (2 doses)

n=1 grade 4 TMA
n=1 grade 3 steroid myopathy

n=1, Grade 4 decrease

Cohort 3: Low-dose pediatric

Pt	Age	Wt
1008	12.3	70.5
1009	11.7	55.7

RP-A501 6.7 x 10^{13} GC/kg

Daily, real-time monitoring of labs & clinical status

Day -14 | Day 14 | Month 3

Steroid (prednisone 0.5 – 0.8mg/kg); Taper start d 10

Sirolimus (target trough 4-8 ng/mL)

Rituximab (2 doses)

none

Remain in normal range

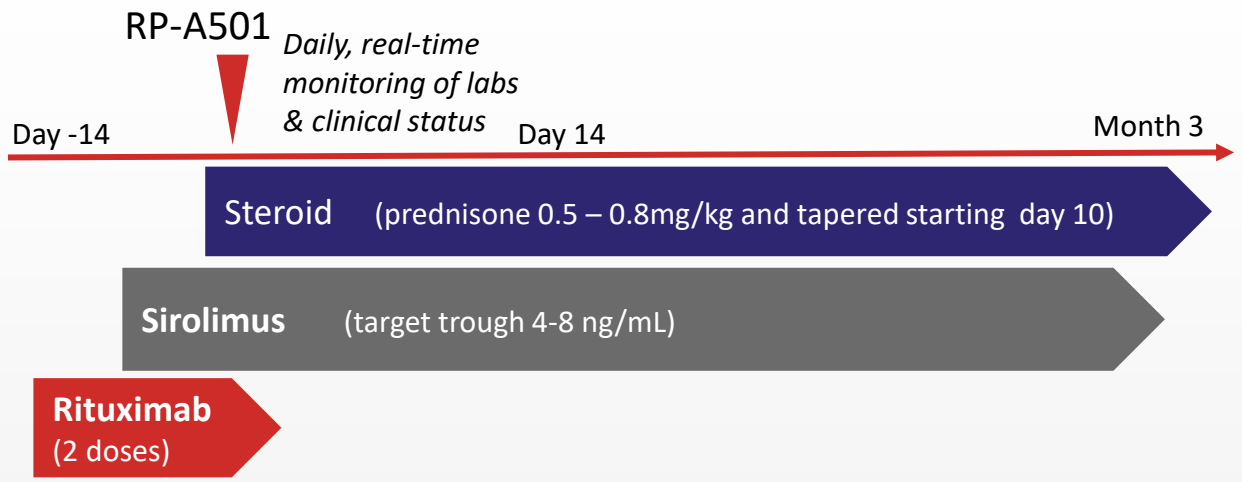
[a] Corticosteroid compliance uncertain
[b] This patient underwent heart transplant due to DD progression five months post RP-A501 infusion; both because this patient would no longer be eligible for future studies based on revised eligibility criteria and because long-term RP-A501 effect is not evaluable, data for this patient are not shown. He is currently stable.

d, day; LLN, lower limit of normal; Pt, patient; SAE, serious adverse event; TMA, thrombotic microangiopathy; Wt, weight.
Age in years at RP-A501 infusion; weight in kg.

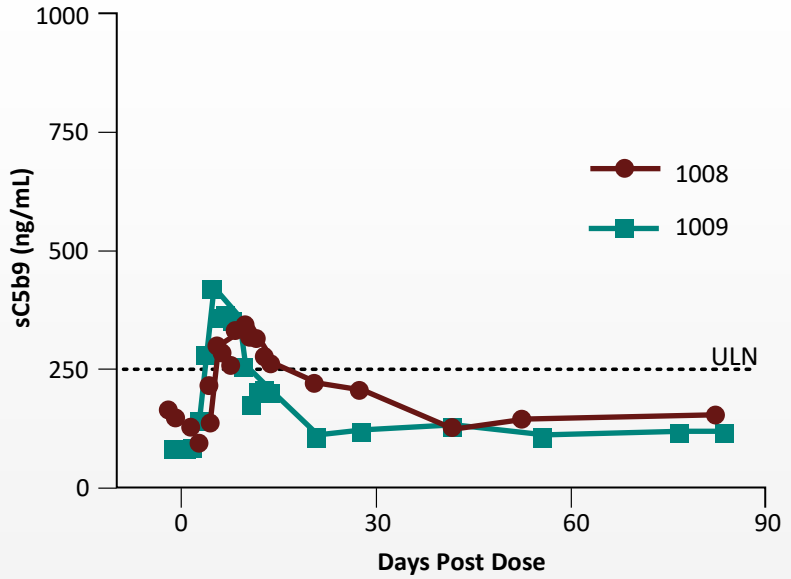
RP-A501 Was Generally Well Tolerated in Pediatric Cohort on Enhanced Immunomodulation

All AEs were Transient and Reversible with 6 and 11 month follow up in 1008 and 1009, respectively

Pediatric immunomodulation protocol



sC5b9 Evaluation Post RP-A501



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; ULN, upper limit of normal.
Data cut-off September 27, 2022 with source data verification through July 11, 2022.

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Early Pediatric Data are Encouraging and Consistent with Adult Efficacy

Patient ID: A501-008-1008

Baseline Characteristics

AGE AT INFUSION
12.3 years

ICD yes[1]

WPW yes

MAX LV WALL THICKNESS
41.9 mm, z-score +32

6MWT
438 meters

9 months

Variable	Baseline[2]	Most Recent Follow-up
LAMP2 protein[3] (%)	0.5	21[4]
Troponin-I (ng/mL)	1.89	0.28
BNP (pg/mL)	1837	406
KCCQ-overall scale	50	93
NYHA Class	II	I

Patient ID: A501-008-1009

Baseline Characteristics

AGE AT INFUSION
11.7 years

ICD no

WPW no

MAX LV WALL THICKNESS
19.8 mm, z-score +12

6MWT
553 meters

6 months

Variable	Baseline[2]	Most Recent Follow-up
LAMP2 protein[3] (%)	2.6	35[4]
Troponin-I (ng/mL)	0.67	0.07
BNP (pg/mL)	297	113
KCCQ-overall scale	52	81[5]
NYHA Class	II	I

[1] Recommended prior to enrollment; ICD implanted 3 months after RP-A501 infusion
[2] Baseline values for troponin-I and BNP are the mean values from all pre-dose visits
[3] All biopsies stained for LAMP2 were compared to normal controls. Data is quantitated in a blinded fashion from ~3-5 sections
[4] Most recent biopsy data available from 6 month visit for 1008 and 3 month visit for 1009
[5] Most recent KCCQ data available from 3 month visit for 1009

6MWT, 6-minute walk test; BNP, brain natriuretic peptide; ICD, implantable cardioverter defibrillator; KCCQ, Kansas City cardiomyopathy questionnaire; LAMP2, lysosome-associated membrane protein 2; LV, left ventricle; NYHA, New York Heart Association; WPW, Wolff-Parkinson-White syndrome.
Data cut-off September 27, 2022 with source data verification through July 11, 2022.

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Early Pediatric LAMP2 Expression are Encouraging and Consistent with Adult Data

Quantified LAMP2 protein expression by immunohistochemistry (IHC)

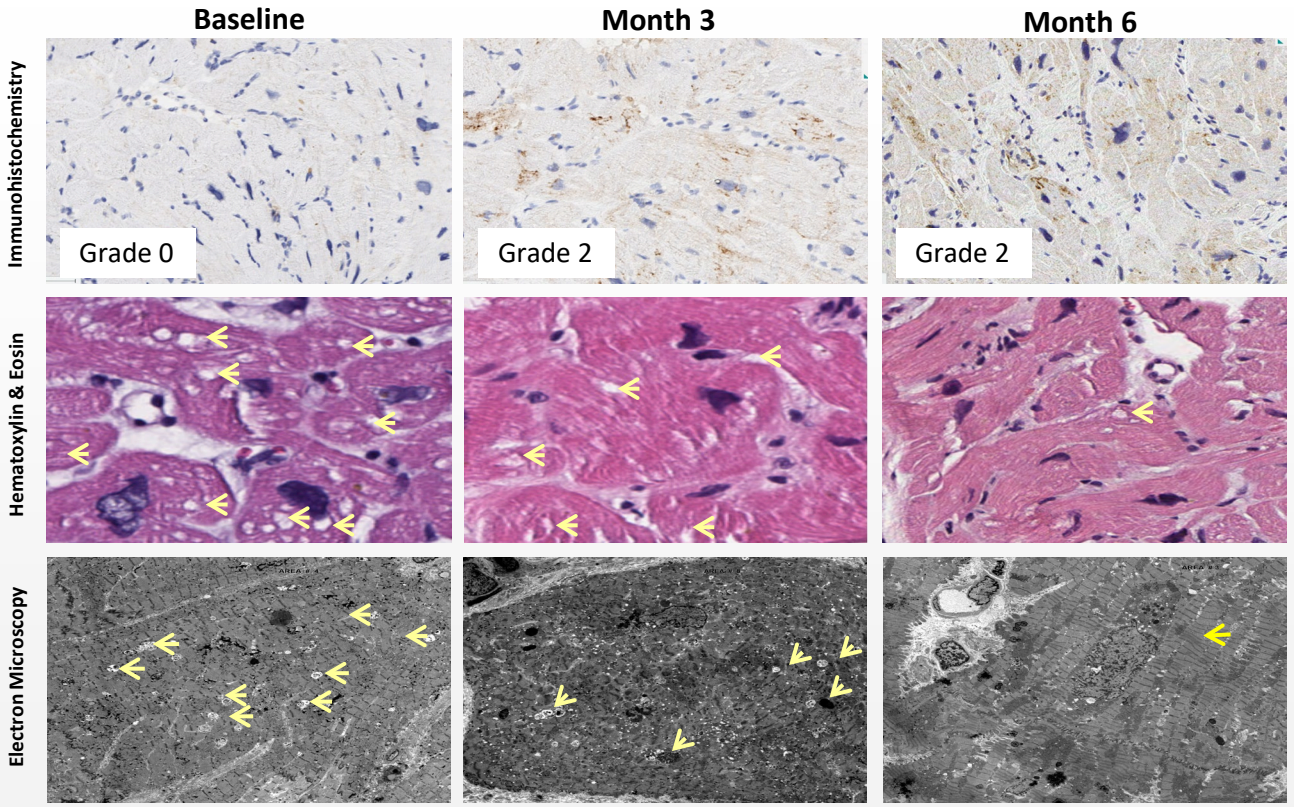
Cohort	Patient ID	Initial Biopsy Post Infusion
Pediatric - Low Dose	**1008**	*Month 3:* **18.5%***
	1009	*Month 3:* **34.7%**
Adult - Low Dose	1001	*Month 2:* 7.3%
	1002	*Month 2:* 36.9%
	1005	*Month 2:* 17.6%
Adult - High Dose	1006	*Month 2:* 5.0%
	1007	*Month 2:* 6.9%

All biopsies stained for LAMP2 were compared to normal control samples. Data is quantitated in a blinded fashion from ~3-5 sections.
* 1008 Month 6 biopsy: 21% as noted in previous slide

LAMP2, lysosome associated membrane protein 2.
IHC, immunohistochemistry.

LAMP2 Myocardial Protein Expression and Histologic Improvement in the Pediatric Cohort

A501-008-1008 Endomyocardial Biopsy (EMB) Images



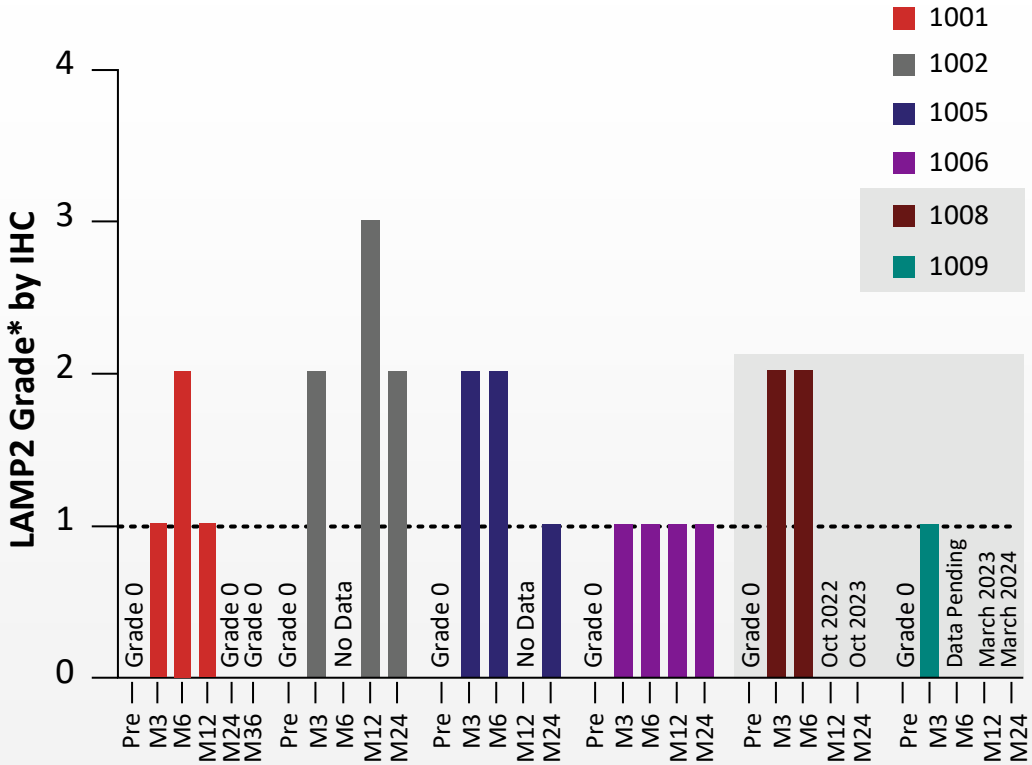
LAMP2 protein expression assessed (relative to normal human controls) by core lab in a blinded fashion of entire tissue sample

- 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%
- H&E images captured at 20x magnification, presented digitally zoomed
- Arrows indicate autophagic vacuoles
- Similar findings on EMB from patient 1009 at Baseline and Month 3

EMB, endomyocardial biopsy; H&E, hematoxylin & eosin stain; LAMP2, lysosome associated membrane protein 2.

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)

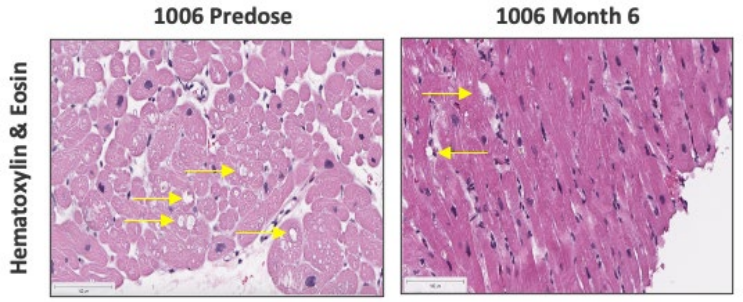
Patient ID	Predose	Month 6	Month 12	Month 36
1001[a]	0	0.384	0.197	0.120
1002	0	ND	0.575	0.590[c]
1005	0	0.583	ND	1.228[c]
1006	0	2.693	1.131	-
1007	0	RV: 6.77[b] LV: 9.15[b]	Post heart transplant	
1008	0	0.492	-	-
1009	0	Data pending	-	-

LV, Left ventricle and RV, Right ventricle at 5 months from explanted heart; ND. not done, -, visit pending.
[a] Corticosteroid compliance uncertain. [b] Assessment from explanted heart tissue at 5 months. [c] Month 30 visit.

LAMP2, lysosome associated membrane protein 2; M, month.

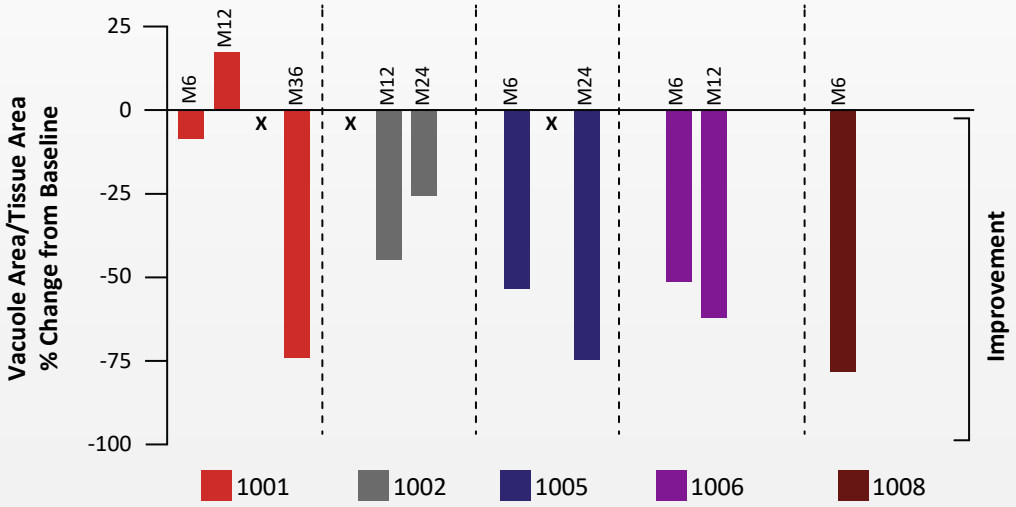
Restored Autophagy is Sustained Following RP-A501

Restored autophagy indicated by attenuation of vacuolar area

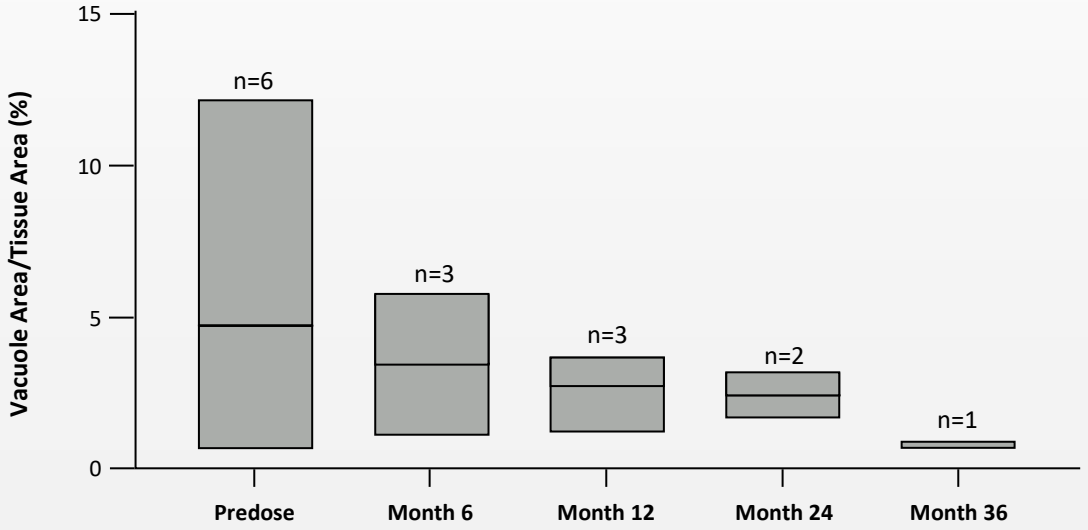


Light microscopy images at 20X;
Autophagic vacuoles are depicted by yellow arrows.

A. Vacuolar Area of Endomyocardial Tissue



B. Vacuolar Area Decreases with Treatment

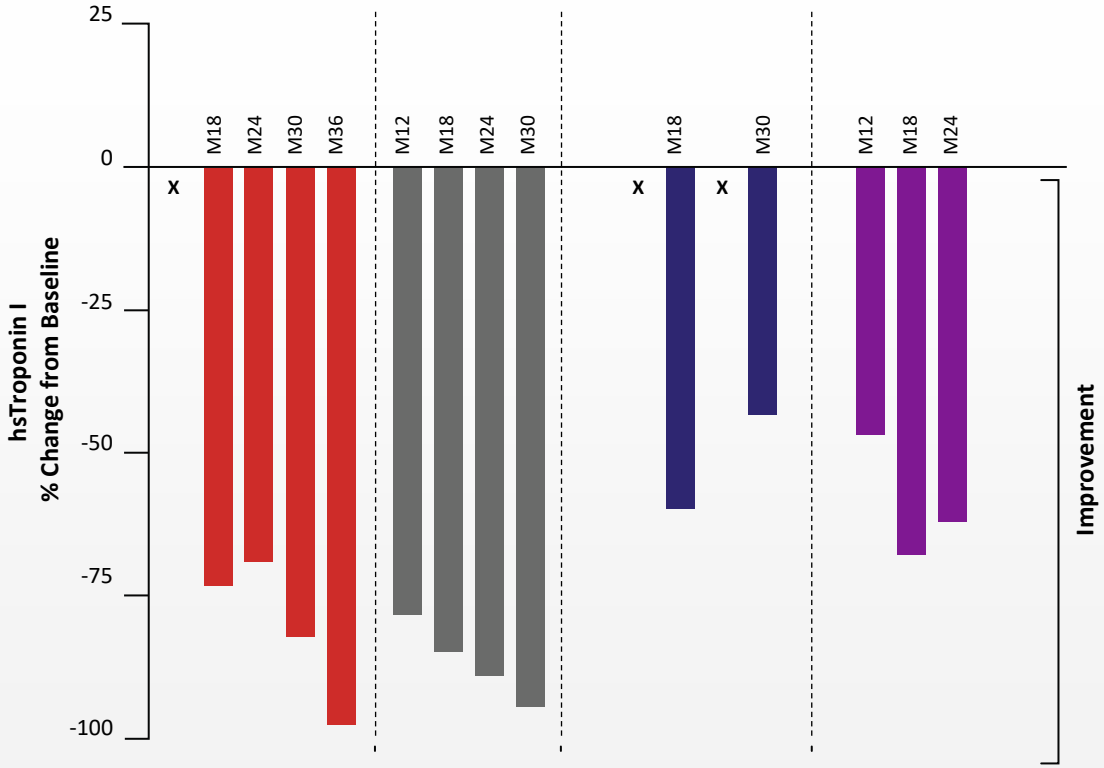


M, month; X, data not assessed

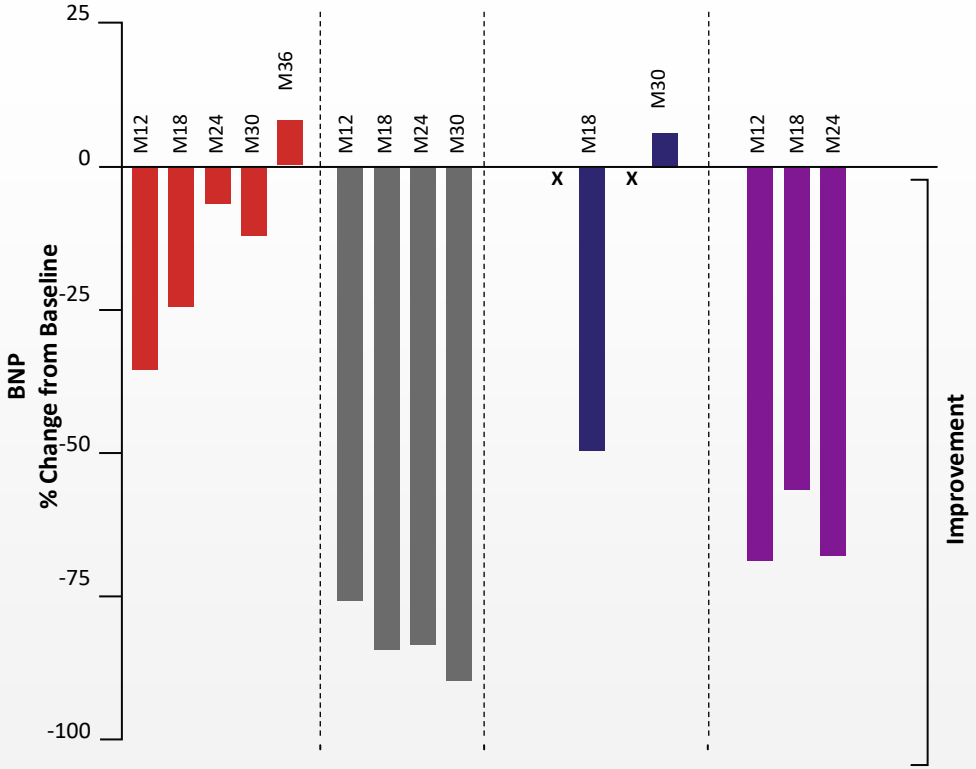
Bars denote minimum and maximum range, and line within each bar represents the mean value within study population.

Sustained Improvement or Stabilization of Biomarkers of Myocardial Injury and Stress Following RP-A501

High Sensitivity Troponin I (hsTnI)



Brain Natriuretic Peptide (BNP)

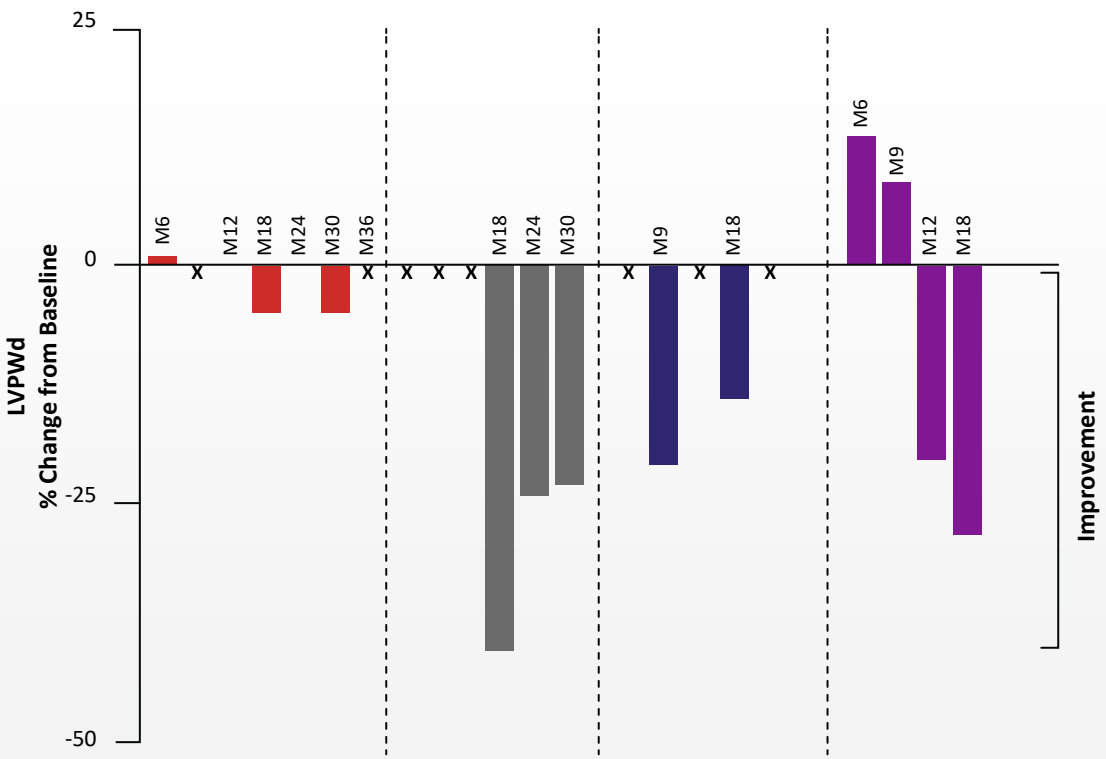


1001 1002 1005 1006

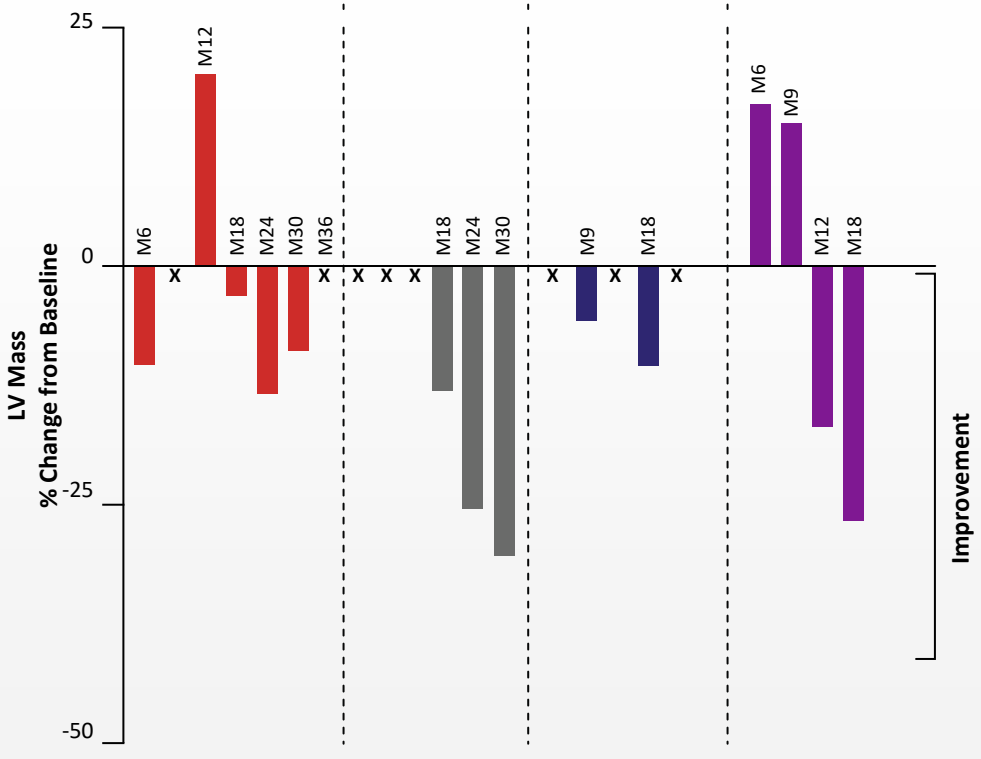
M, month; X, data not assessed

Sustained Improvement or Stabilization of LV Hypertrophy Following RP-A501

LV Posterior Wall Thickness



LV Mass



1001 1002 1005 1006

LV, left ventricle; LVPWd, LV posterior wall end diastole; M, month; X, data not assessed
LV hypertrophy is assessed via LV posterior wall at end diastole (LVPWd) and LV mass.

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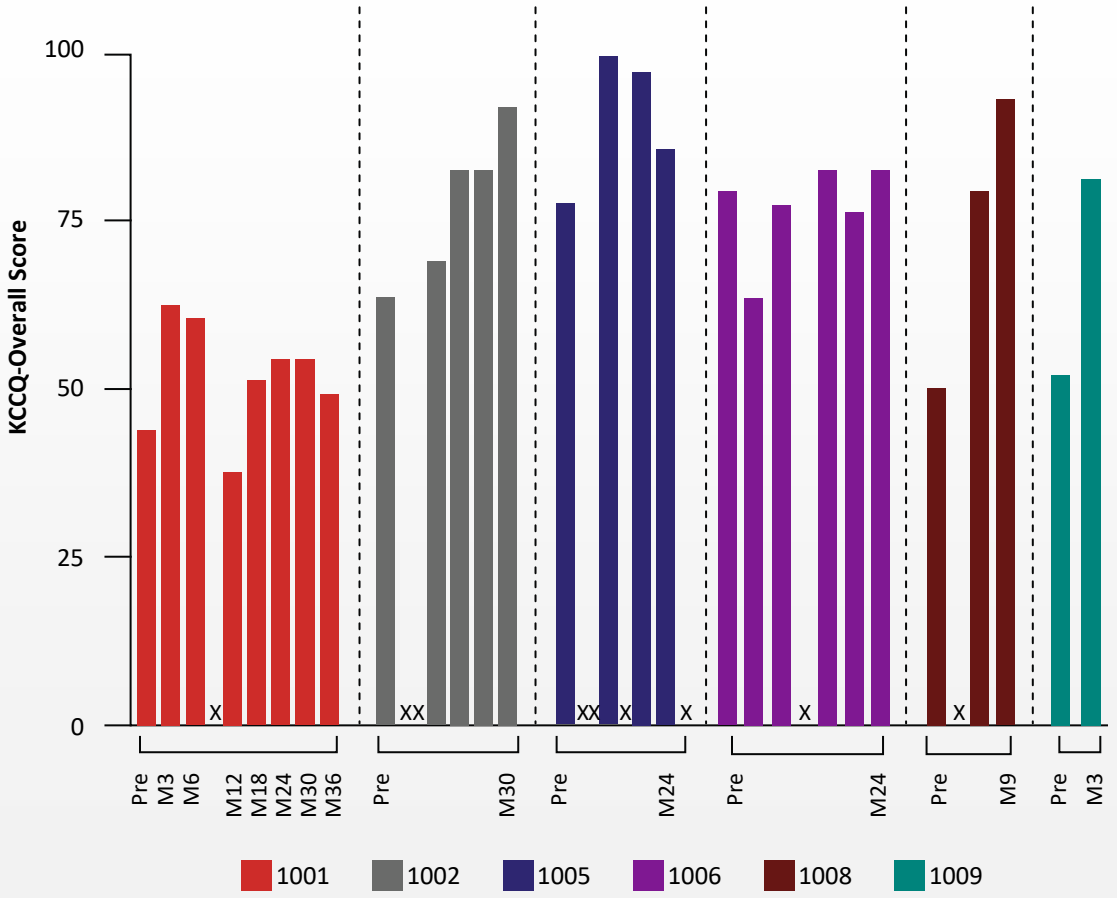
Sustained Improvement or Stabilization of Functional Cardiac Status Following RP-A501

New York Heart Association Class

Cohort	Patient ID	Baseline	Month 12	Most Recent Follow-up	Time of Most Recent Follow-up
Low Dose Adult	1001[a]	II	II	II	36 months
	1002	II	III	I	30 months
	1005	II	II	I	30 months
High Dose Adult	1006	II	I	I	24 months
Low Dose Pediatric	1008	II	October 2022	I	9 months
	1009	II	March 2023	I	6 months

Kansas City Cardiomyopathy Questionnaire Overall Score

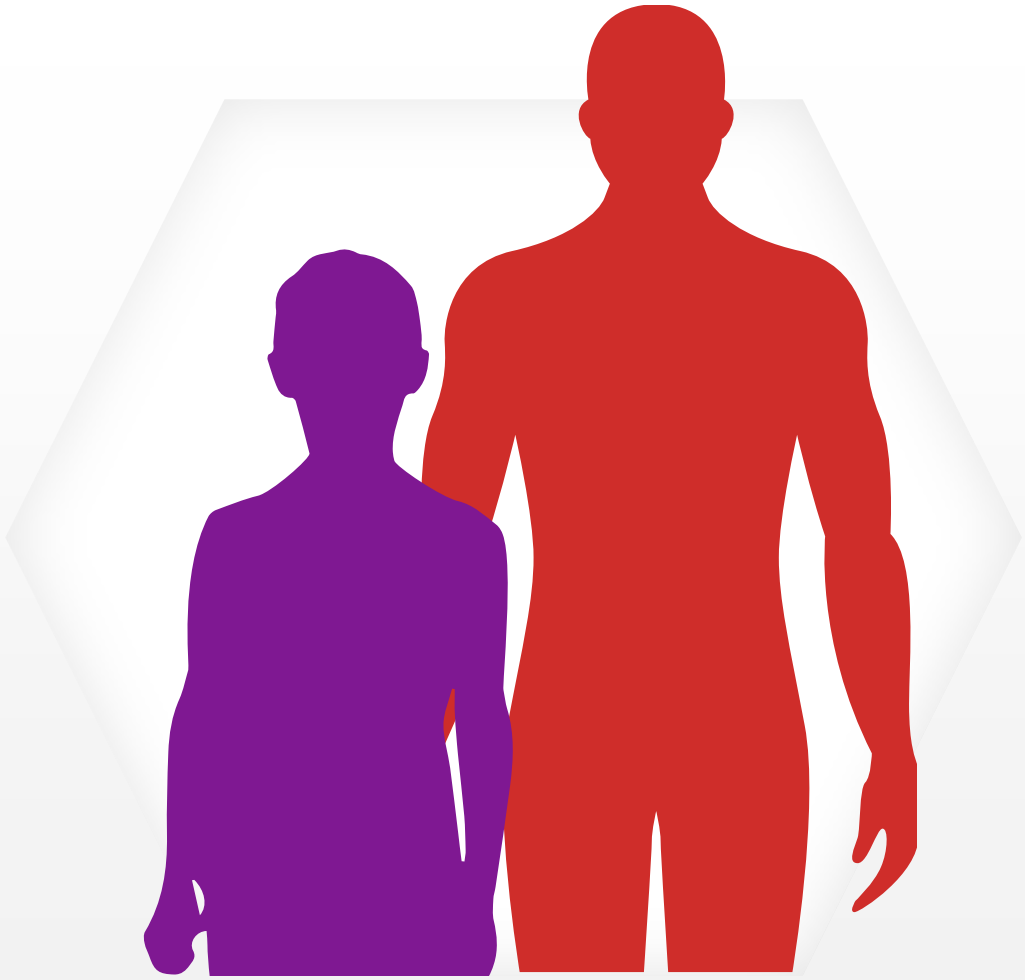


M, month; X, data not assessed.
[a] Corticosteroid compliance uncertain

Summary of Results and Conclusions

PEDIATRIC COHORT

- RP-A501 was well tolerated
- No immediate, early or delayed RP-A501 related SAEs observed to date with enhanced immunomodulation
  - Minimal complement activation
  - Platelets remained within normal range
- Absent or limited worsening of skeletal myopathy with reduced steroid dose and more rapid taper, and introduction of sirolimus
- Increased LAMP2B protein expression was associated with early signals of improved cardiac histology, as well as serological evidence of decreased myocardial injury and stress
- Early improvement in NYHA class and KCCQ for both patients



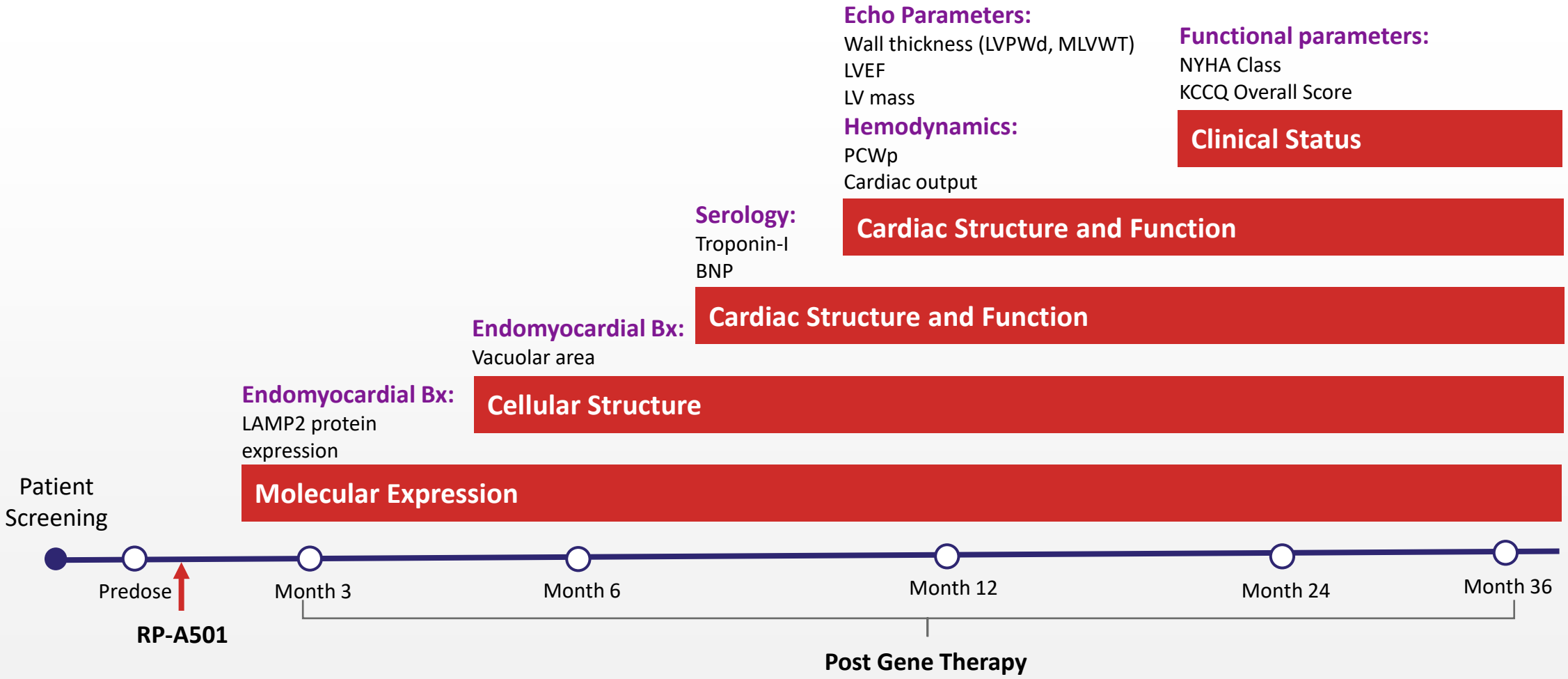
ADULT COHORT

- Low-dose continues to be generally well tolerated at 2-3 years post-treatment
- Increased LAMP2B protein expression was associated with durable disease improvement or stabilization including clinical status (NYHA class, KCCQ), LV hypertrophy (LV wall thickness and mass), biomarkers of myocardial injury and stress (hsTroponin I and BNP), and cardiac histology
- All patients are alive and well in their early 20s, whereas median survival in DD males is 19 years old*

BNP, brain natriuretic peptide; hs, high sensitivity; KCCQ, Kansas City cardiomyopathy questionnaire; LAMP2B, lysosome-associated membrane protein 2B; LV, left ventricle; NYHA, New York Heart Association; SAE, serious adverse event.

*Except for patient 1007 who received a heart transplant at 5 months due to Danon Disease progression. He is currently stable. Brambatti M et al. *Int J Cardiol.* 2019;286:92-98.

Connecting Surrogate Endpoints to Functional Outcomes for Pivotal Study*



BNP, brain natriuretic peptide; Bx, biopsy; KCCQ, Kansas City cardiomyopathy questionnaire; LAMP2, lysosome-associated membrane protein 2; LV, left ventricle; LVEF, LV ejection fraction; LVPWd, LV posterior wall end diastole; MLVWT, maximal LV wall thickness; NYHA, New York Heart Association; PCWp, pulmonary capillary wedge pressure.
*Pending regulatory feedback

Summary of Results and Conclusions

Phase 1 enrollment and treatment are **complete**

- ✓ The enhanced immunomodulatory regimen **was well tolerated and has effectively mitigated adverse events** in the pediatric cohort, who are currently 6 to 11 months post treatment
- ✓ The **early LAMP2 expression data from the pediatric cohort are encouraging and consistent** with that seen in the adult patients at the same timepoints
- ✓ The **early clinical trends for the pediatric cohort are encouraging and consistent** with the **sustained clinical responses seen in the adults at 24-36 months**
- ✓ **Study design and endpoints have been identified** for the planned Phase 2 pivotal study* and **endorsed by an International Scientific and Clinical Advisory Board**; FDA discussion planned at the end of this year

*Pending regulatory feedback

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

- 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

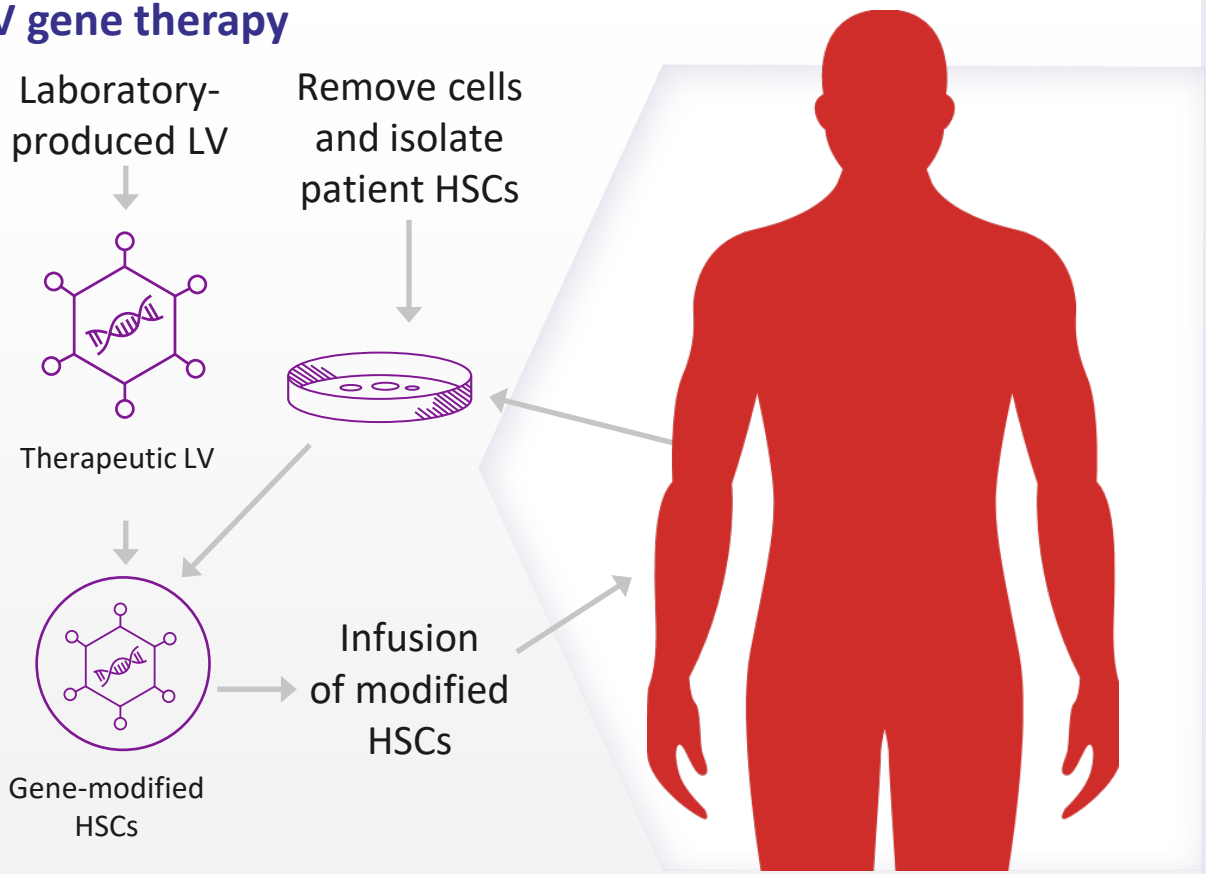
IDEAL FOR

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

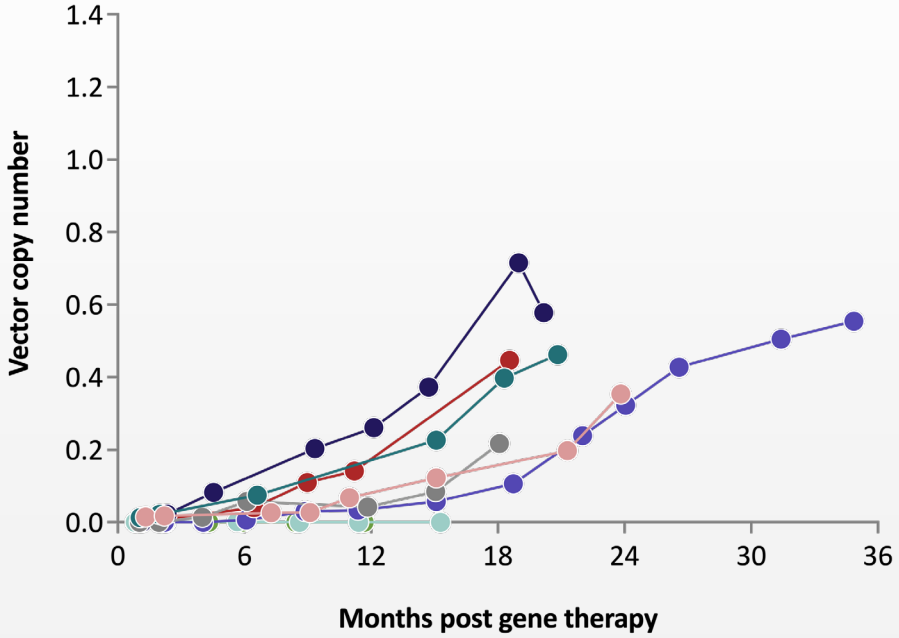
*Efficacy in ≥5 patients (observed over >1 year post prescription) required to reject null hypothesis. † In absence of conditioning, ≥12 months follow-up required to identify engraftment and MMC-resistance.
BM CFC, bone marrow colony forming cell; BMF, bone marrow failure; FA, Fanconi Anemia; HSC, hematopoietic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

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

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

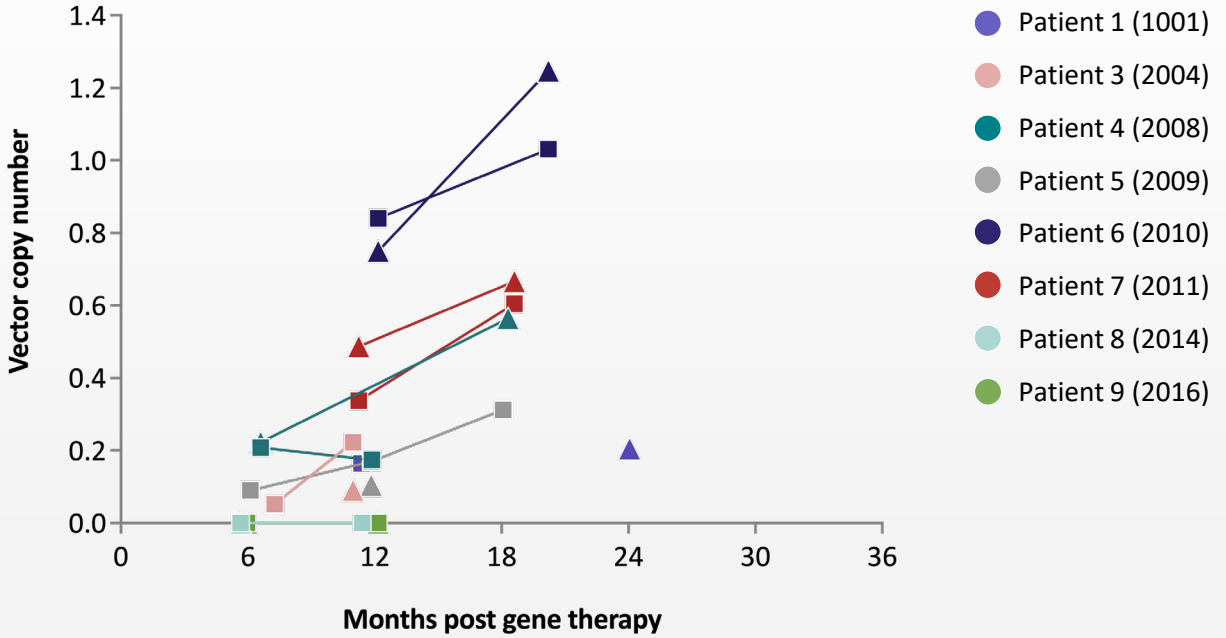
Peripheral blood VCN

○ VCN in peripheral blood mononuclear cells



Bone marrow VCN

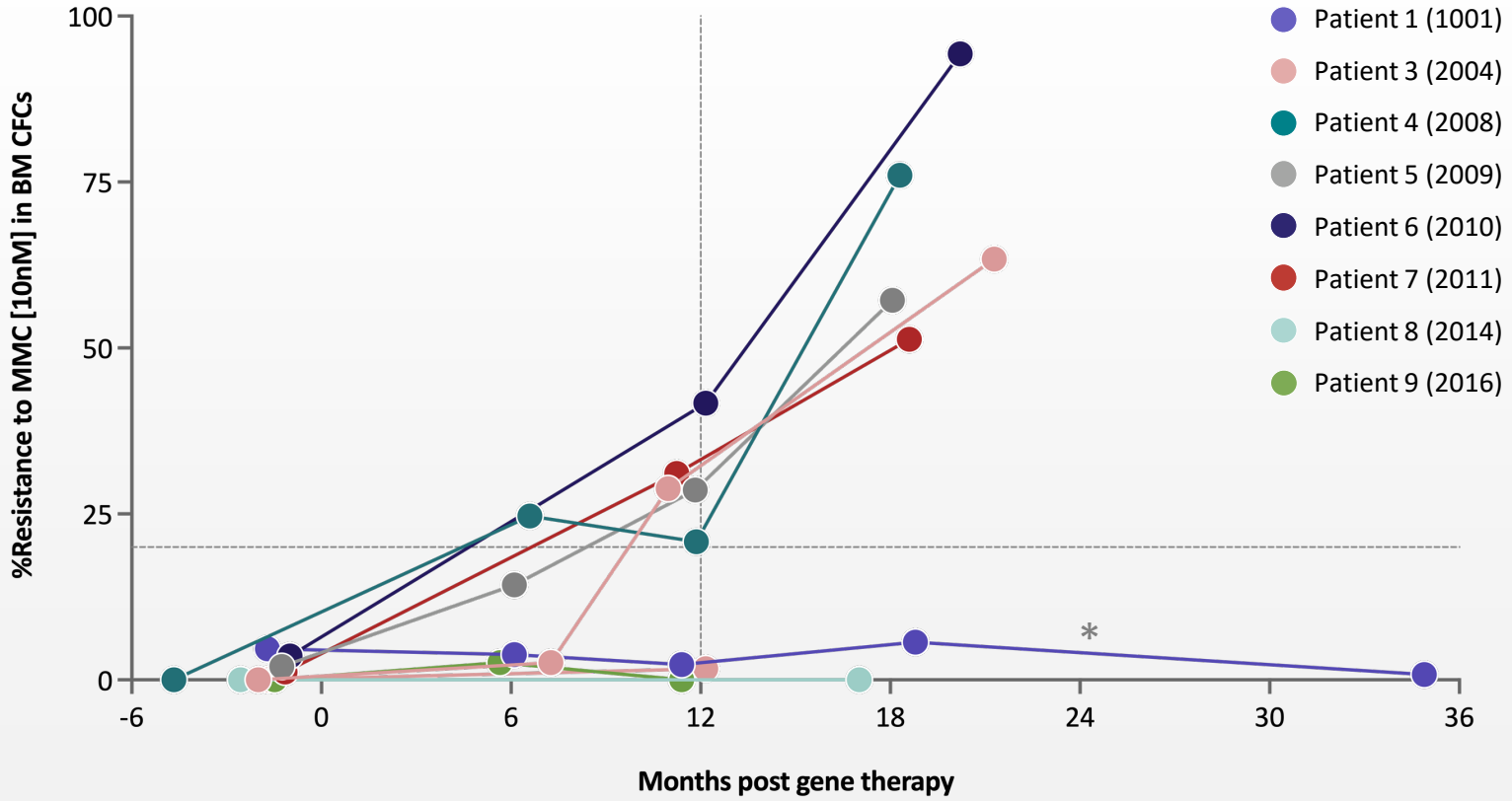
□ VCN in bone marrow mononuclear cells
△ VCN in bone marrow CD34+ cells



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

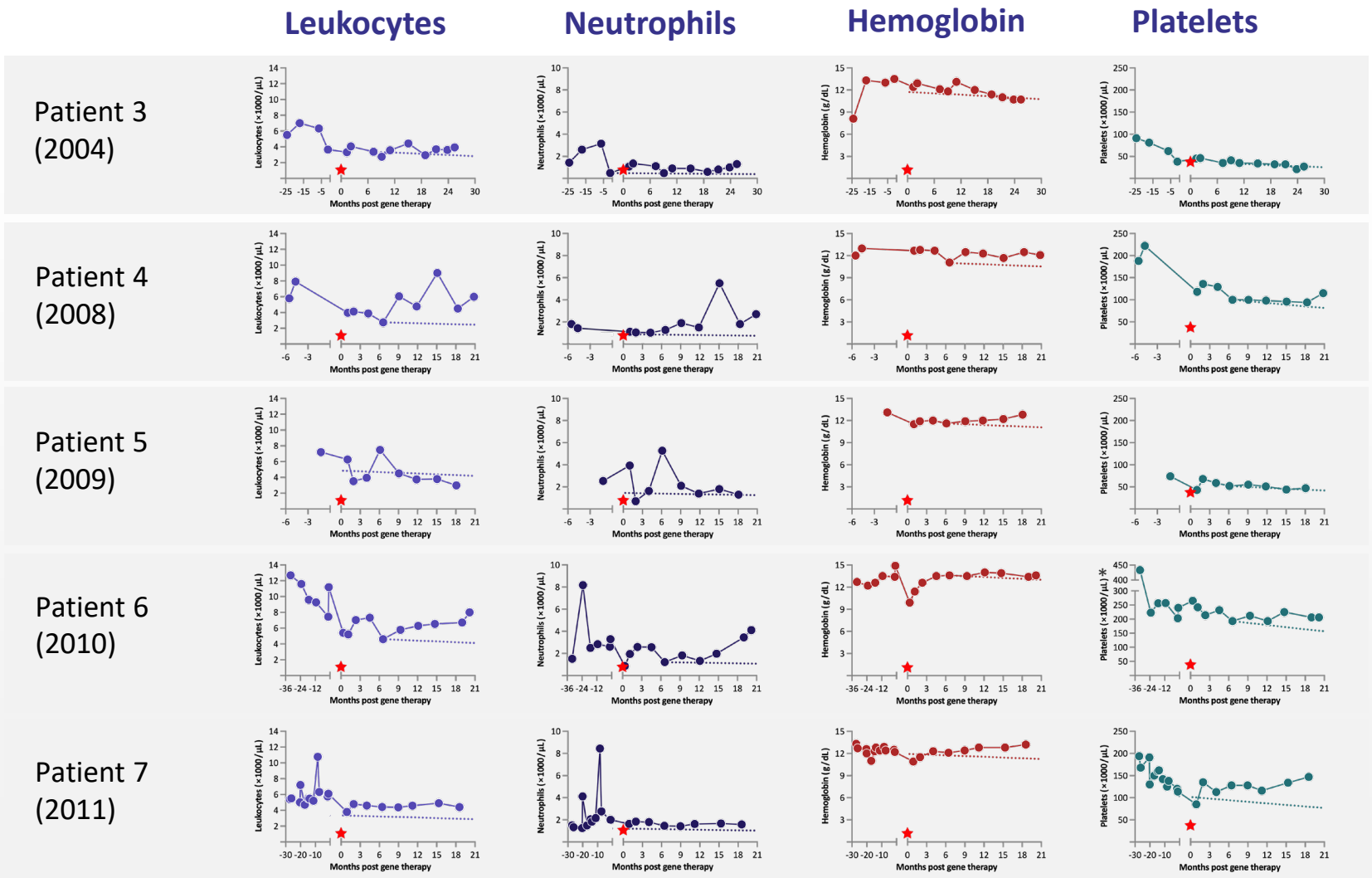


For 5 patients, increased BM CFC MMC resistance ranging from 51% to 94% observed at 18 to 24 months post–RP-L102 administration

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

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.

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

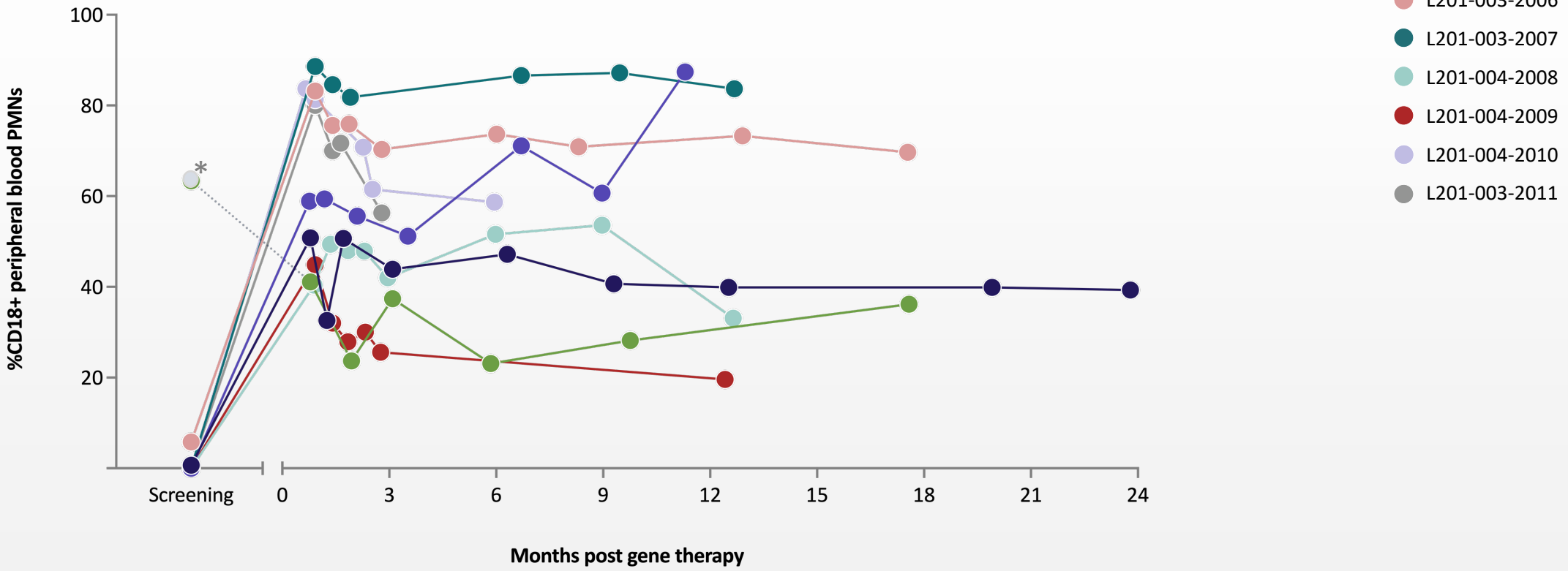
GvHD, graft-versus-host disease; HSC, hematopoietic stem cell; ISA, Insertion site analysis; LAD-I, Leukocyte Adhesion Deficiency-I; PMN, polymorphonuclear neutrophils; SAE, serious adverse event; VCN, vector copy number.

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**

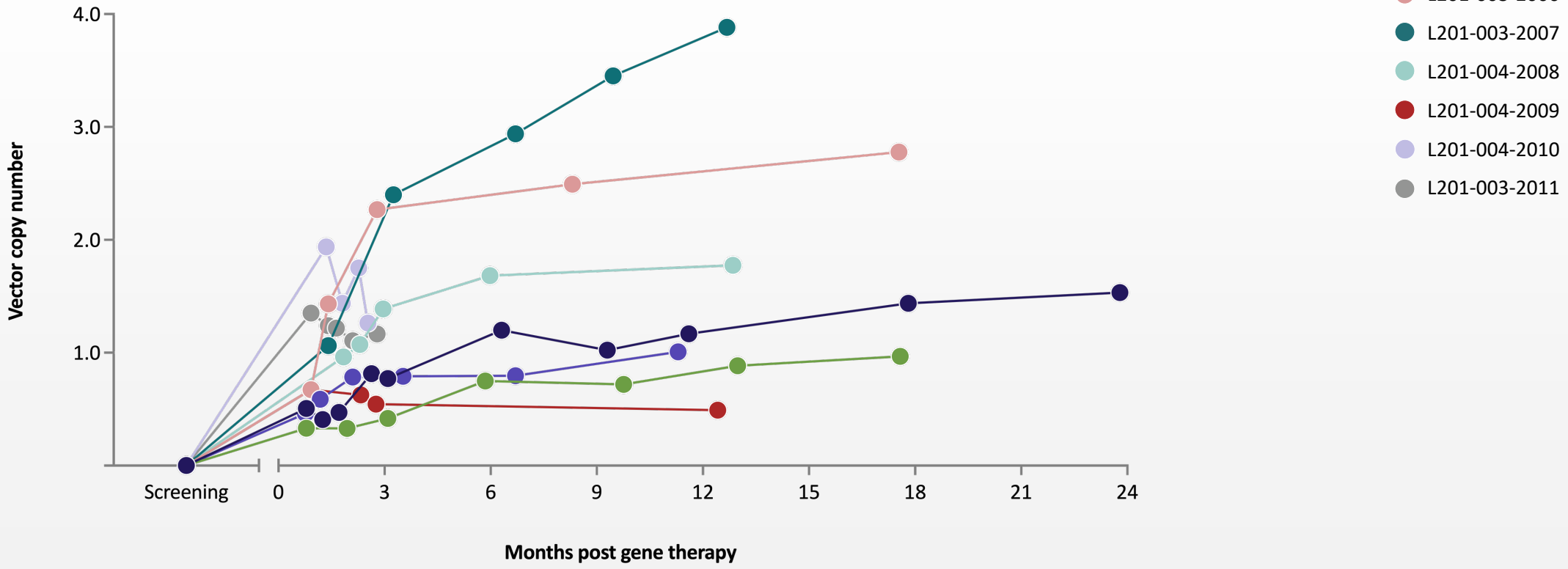
At 3 to 24 months after infusion, 9/9 patients sustained stable CD18 expression (median: 56%) with no therapy-related serious adverse events



*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**

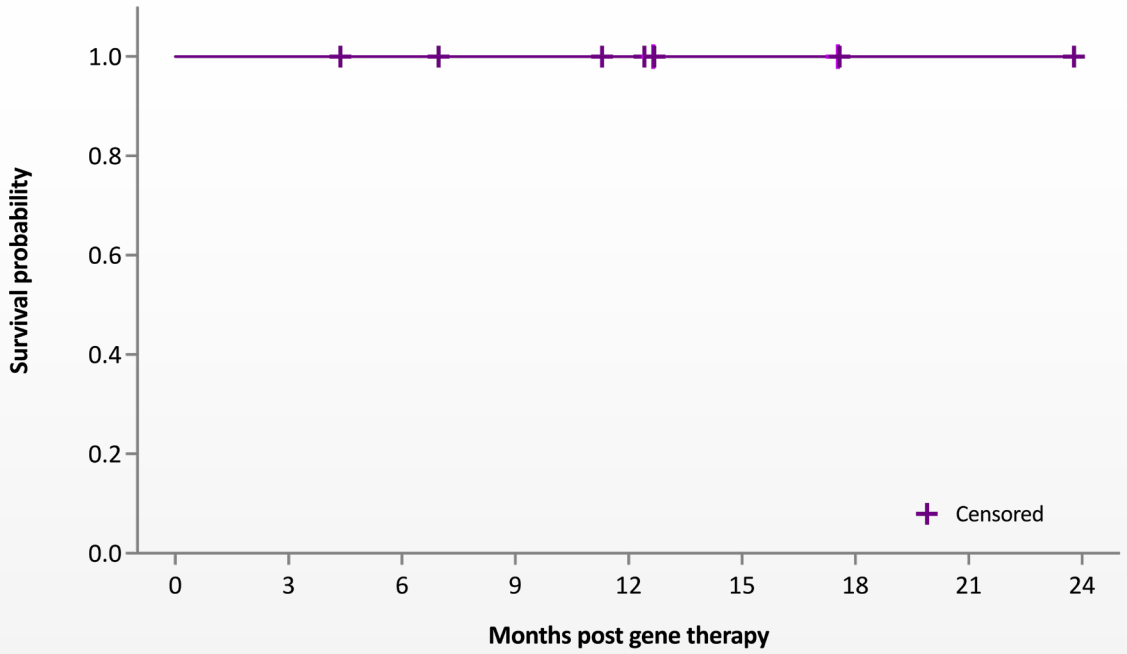
At 3 to 24 months after infusion, CD18 expression is accompanied by stable PB VCN (range: 0.5 – 3.9; median: 1.2 copies per PB mononuclear cell)



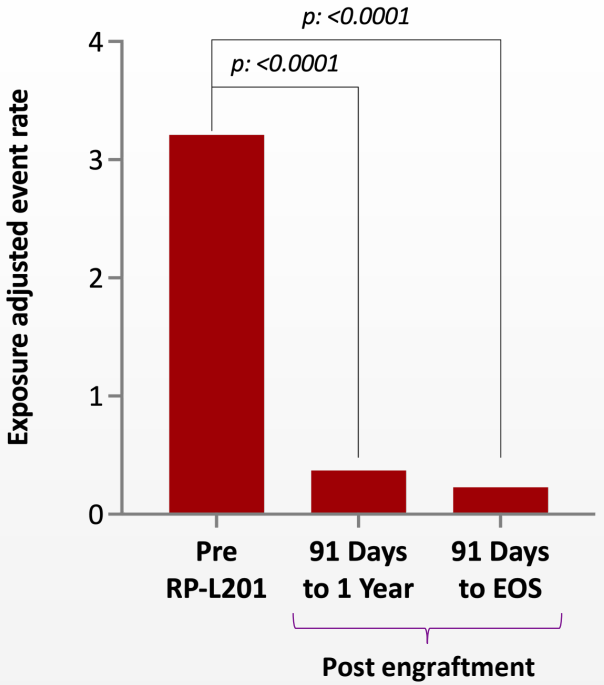
LAD-I, Leukocyte Adhesion Deficiency-I; PB, peripheral blood; PBMCs, peripheral blood mononuclear cells; VCN, vector copy number.
Data on file. Rocket Pharmaceuticals. 2022. Data Cut-Off: March 9, 2022.

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

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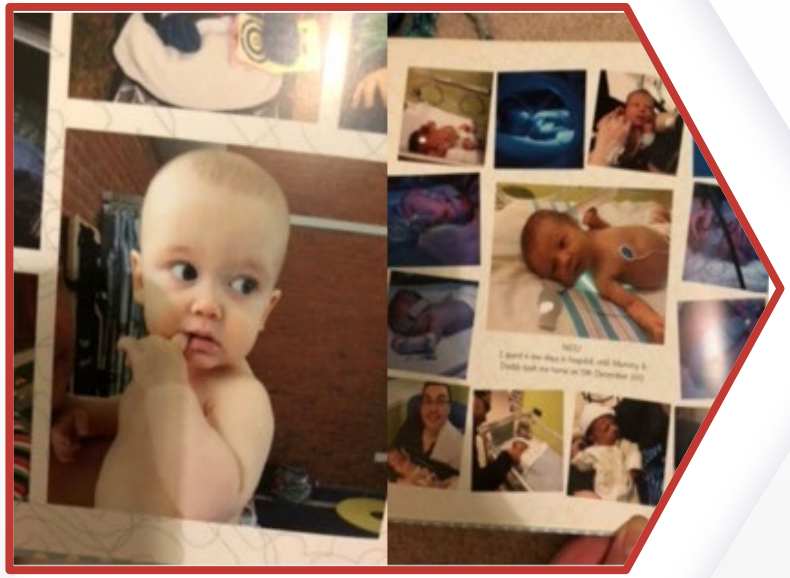
RP-L301 for PKD: ***PKLR*** Gene Mutation



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

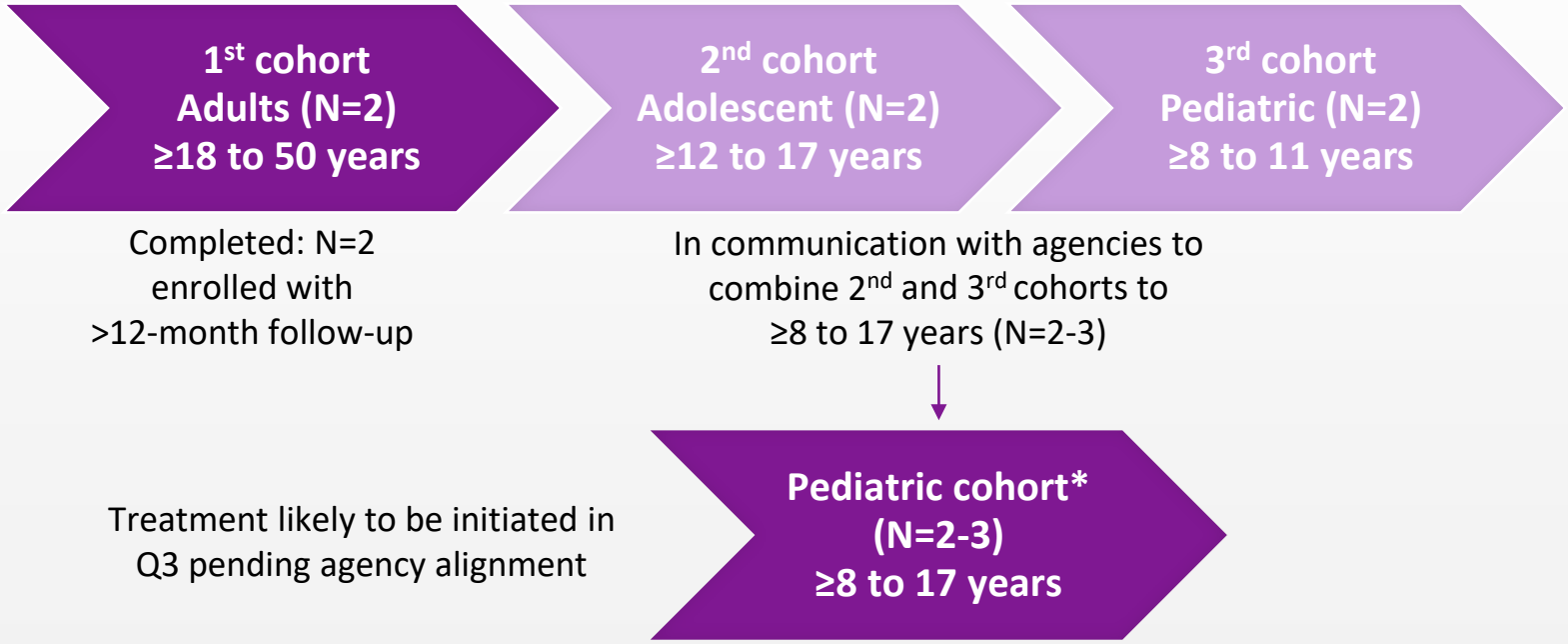
Selected secondary endpoints:

- 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.

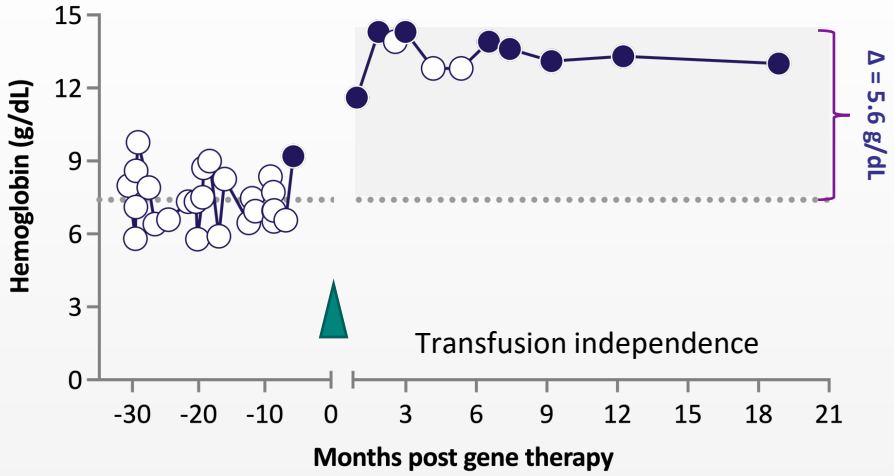
HSC, hematopoietic stem cell; IP, investigational product; LV, lentiviral vector; PKD, Pyruvate Kinase Deficiency; Q2, second quarter of the year; SAE, serious adverse event.

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

Hemoglobin Normalization and Transfusion Independence

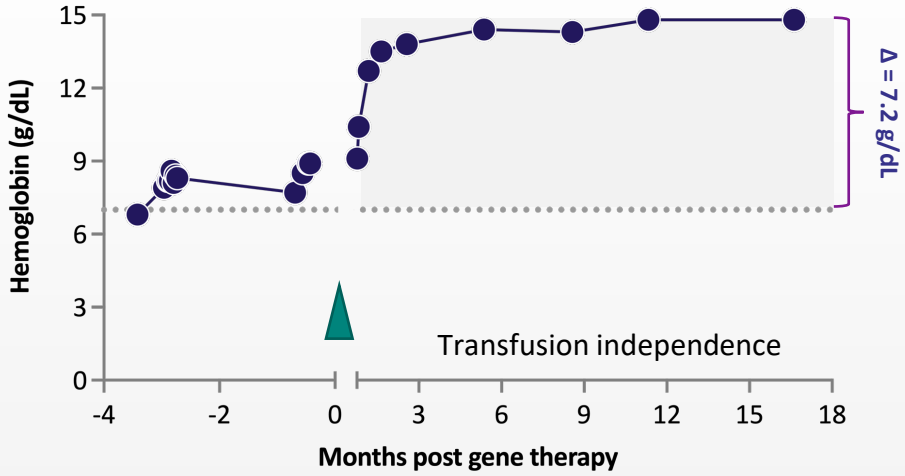
Hemoglobin improvement to normal range (from baselines in severe (<8g/dL range)
Transfusion independence (extensive transfusion requirements prior to RP-L301)
Sustained improvement of hemolysis markers (LDH, bilirubin) and PB VCNs in 1.0 – 3.0 range

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

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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)

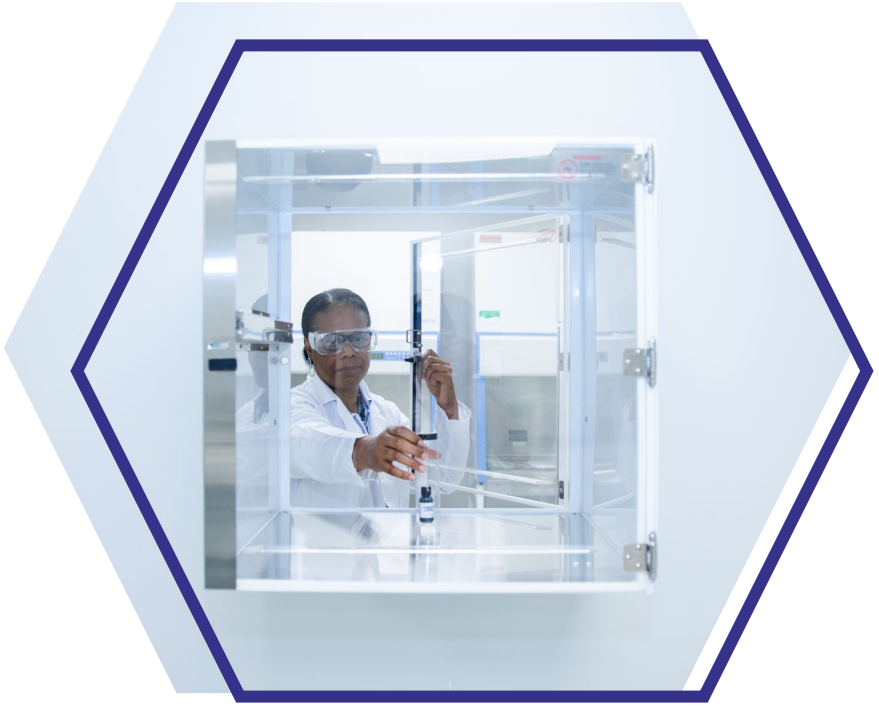
LIFE-CYCLE MANAGEMENT

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



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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

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.

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

- FDA EOP1 agreement and initiation of Phase 2 pivotal study activities
- FA: Guidance on BLA filing timeline
- PKD: Phase 1 adult cohort data

2023

- LAD-I: BLA/MAA filings in 1H’23
- FA: BLA/MAA filings in 2H’23
- PKD: Phase 1 update in 1H’23 and initiation of Phase 2 pivotal study in 2023
- 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.

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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