40th Annual J.P. Morgan Healthcare Conference
Rocket Pharmaceuticals Company Presentation

Gaurav Shah, MD
Chief Executive Officer

January 10, 2022

rocket pharma

SEEKING GENE THERAPY CURES

NASDAQ: RCKT

Cautionary Statement Regarding Forward-Looking Statements

Various statements in this release concerning Rocket's future expectations, plans and prospects, including without limitation, Rocket's expectations regarding its 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), Infantile Malignant Osteopetrosis (IMO) and Danon Disease, and the safety, effectiveness and timing of related pre-clinical studies and clinical trials, may constitute forward-looking statements for the purposes of the safe harbor provisions under the Private Securities Litigation Reform Act of 1995 and other federal securities laws and are subject to substantial risks, uncertainties and assumptions. You should not place reliance on these forward-looking statements, which often include words such as "believe," "expect," "anticipate," "intend," "plan," "will give," "estimate," "seek," "will," "may," "suggest" or similar terms, variations of such terms or the negative of those terms. Although Rocket believes that the expectations reflected in the forward-looking statements are reasonable, Rocket cannot guarantee such outcomes. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including, without limitation, Rocket's ability to monitor the impact of COVID-19 on its business operations and take steps to ensure the safety of patients, families and employees, the interest from patients and families for participation in each of Rocket’s ongoing trials, our expectations regarding 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, 2020, filed March 1, 2021 with the SEC. 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.

rocket pharma

Rocket Pharma: Seeking Gene Therapy Cures

Multi-Platform Gene Therapy Company

4 of 4 programs w/ compelling clinical proof of concept

First, Best and Only-in-Class

Clear MOAs and **clinical endpoints**

Strong Commercial and Manufacturing Capabilities

Value-based and cost-effective pricing strategy; targeting fatal and serious diseases with ***expensive and risky transplants & lifelong treatments as SoC***

In-house AAV manufacturing with capabilities to support commercial product

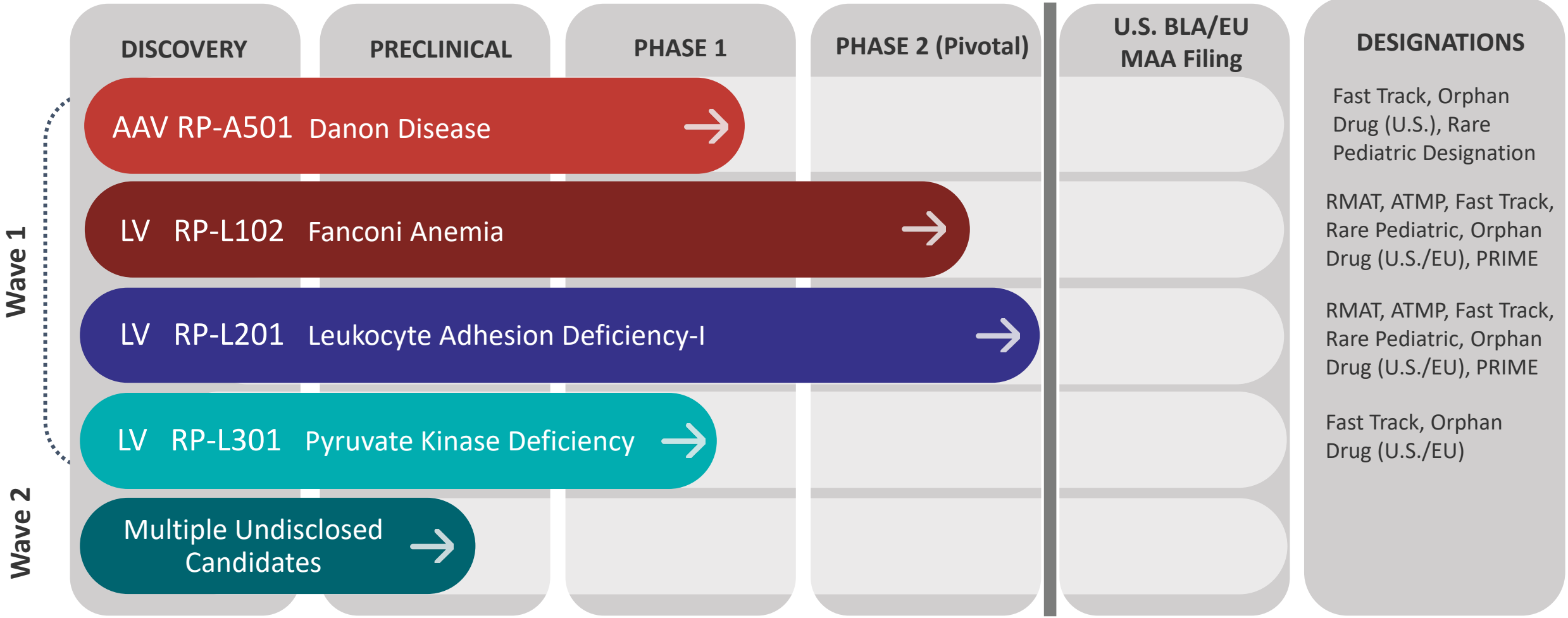
~100,000 sq. ft. facility in Cranbury, NJ

Expert Leadership Team

Seasoned leadership team with a track record of 20+ drug approvals and launches

rocket pharma

Our Multi-Platform Pipeline: Potential for Significant Value Creation Near and Long Term



Note: IMO Program (RP-L401) to be returned to academic sponsors, human and financial resources to be reallocated to building Wave 2 and accelerating Wave 1

Danon Disease:
A Monogenic Heart Failure Syndrome

RP-A501
Danon Disease

RP-L301
Pyruvate Kinase Deficiency

RP-L102
Fanconi Anemia

RP-L201
Leukocyte Adhesion Deficiency-I

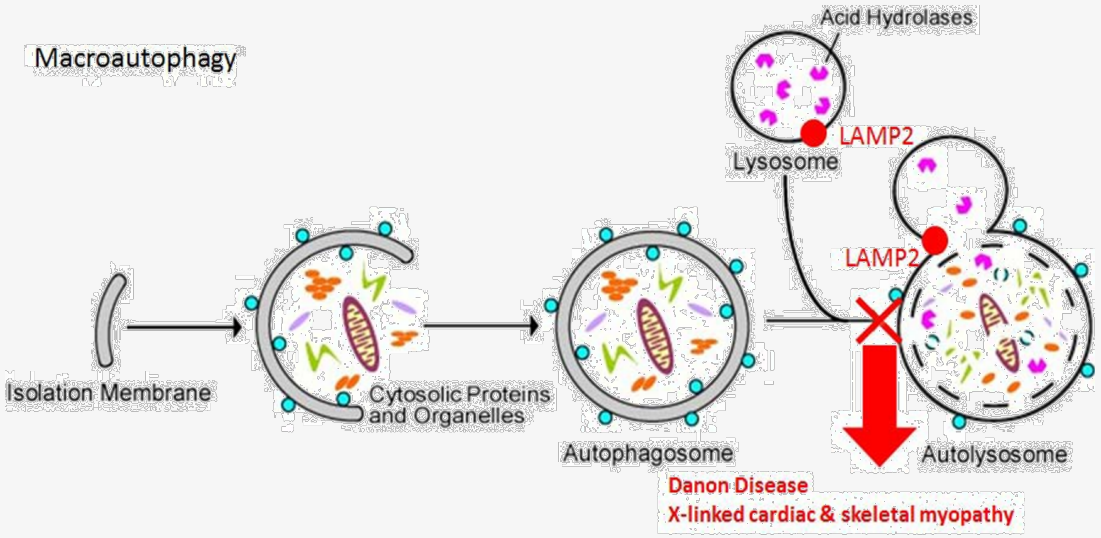
Background: ***Multisystemic disorder*** caused by highly penetrant, X-linked dominant ***LAMP2 mutations;*** progressive cardiomyopathy is predominant cause of early mortality in young adults

- Males: *aggressive* disease course, median OS is 19y
- Females: *generally delayed* presentation due to additional X chromosome

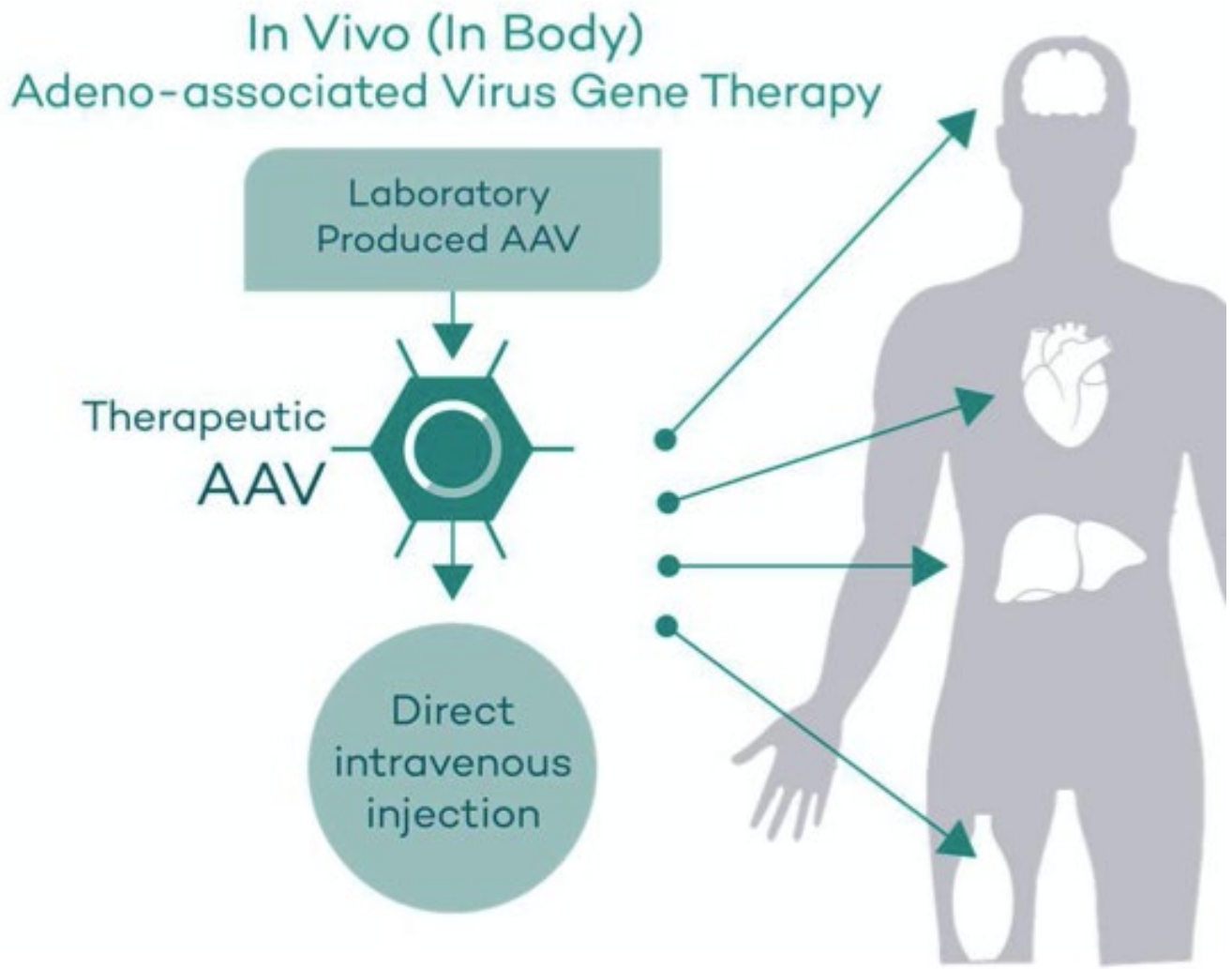
Currently available treatments: Heart transplants associated with considerable morbidity and mortality (10y OS 50%) and available to ~20% of DD pts

Addressable Market: Estimated US + Europe prevalence of ***15,000-30,000***

MECHANISM OF ACTION



How Does rAAV Gene Therapy (RP-A501) Work in Danon Disease?



Intravenous Administration of rAAV

- AAV9 demonstrates tropism to:
  - Cardiomyocytes
  - Skeletal muscle
  - Brain tissue
- Non-dividing, terminally differentiated cardiomyocytes can be transduced
- rAAV9 DNA expresses LAMP2B gene
- Cardiomyocytes have minimal cell turnover; long-term durable expression anticipated

rocket pharma

RP-A501 (Danon Disease): Phase 1 Patients Treated to Date

Non-Randomized Open Label Phase 1 Study (n= 7-9)

✓ **Low Dose Adult**
Age ≥15 years
$6.7x10^{13}$ GC/kg
n=3

Patient ID	Age at Treatment (years)	Weight (kg)	Primary Prophylactic Immunosuppression
1001	17	52.2	Corticosteroids
1002	20	89.1	Corticosteroids*
1005	18	91.8	Corticosteroids*

✓ **High Dose Adult**
Age ≥15 years
$1.1x10^{14}$ GC/kg
n=2

Patient ID	Age at Treatment (years)	Weight (kg)	Primary Prophylactic Immunosuppression
1006	21	82.7	Rituximab + Corticosteroids + Tacrolimus
1007	20	96.7	Rituximab + Corticosteroids + Tacrolimus

→ **Low Dose Pediatric**
Age: 8-14 years
$6.7x10^{13}$ GC/kg
n=1

Patient ID	Age at Treatment (years)	Weight (kg)	Primary Prophylactic Immunosuppression
1008	12	69.7	Rituximab + Corticosteroids + Sirolimus

GC = Genome copies

* Received tacrolimus but after IP dose
1002: For only 5 days between Day 57 and 65 with subtherapeutic levels
1005: Started on Day 10

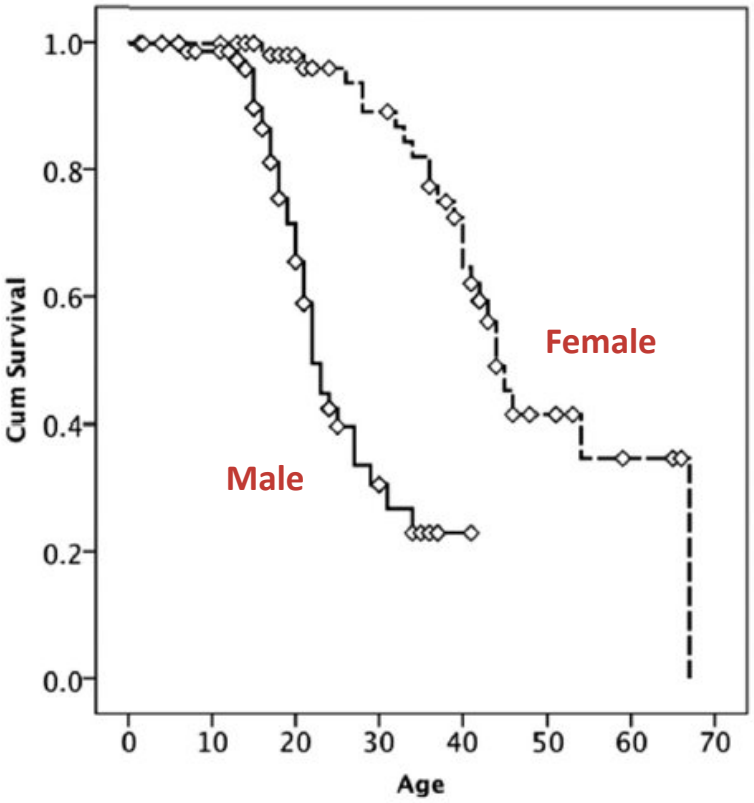
rocket pharma

RP-A501 (Danon Disease): Current Program Safety & Efficacy Data

- Safety data in initial pediatric patient suggests that modified immunosuppressive regimen has potential to mitigate AEs observed in adult low/high-dose cohorts
  - Sirolimus/Rituximab (+ steroid) → :
    - Minimal complement activation and ↓↓ potential for TMA
    - Early steroid taper; no exacerbation of DD-associated skeletal myopathy

- Efficacy in low-dose adult cohort evidenced by multiple clinical parameters in patients with closely monitored immunosuppressive regimen:
  - Improved ventricular wall thickness
  - Improved NYHA class
  - ↓BNP
  - > 50% LAMP2B cardiac expression
  - Stable 6MWT
  - Stable cardiac function as measured by cardiac output / stroke volume and wedge pressure

Danon Disease Natural History

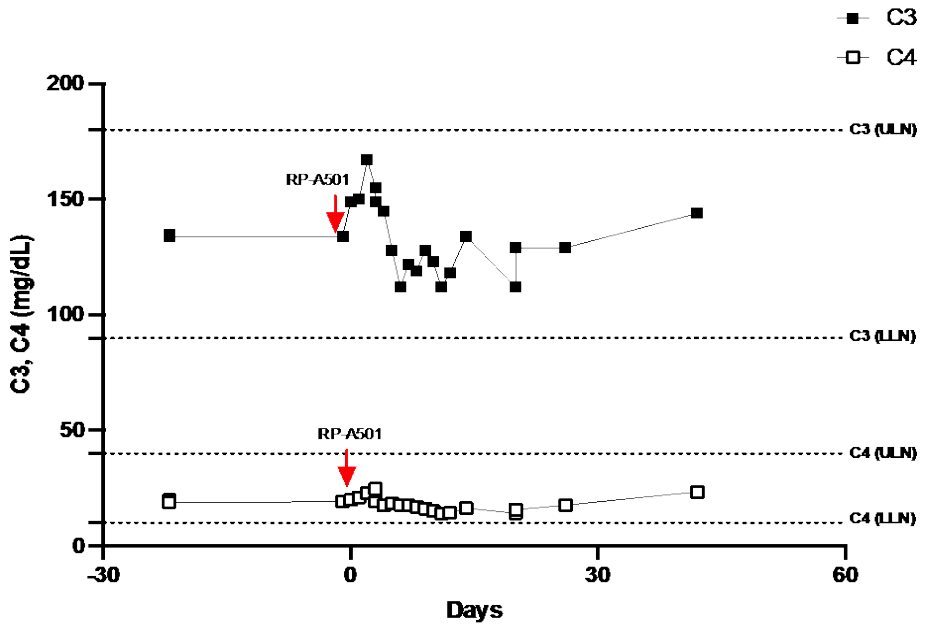


Boucek Genet Med. 2011

RP-A501 (Danon Disease): Pediatric Patient (1008) Safety Data Summary

Post-implementation of enhanced risk-management plan

- RP-A501 infusion was well tolerated
- No drug-related SAEs
- Mitigation of complement activation with stable platelets, Hgb and Cr
- Baseline myopathy (Grade 1) remained stable without exacerbation post-treatment
- Patient has been clinically stable; no signs/symptoms beyond pre-rx baseline
- Additional pediatric patient enrollment on track



RP-A501: Stabilization or Improvement of Cardiac Biomarkers and Functional Status Across Dose Levels

Cohort	Patient ID	Variable	Baseline	Most Recent Follow-up	Time of Follow-up
Adult - Low Dose	1001*	NYHA class	II	II	24 months
		BNP (pg/mL)	70	30	
		6 MWT (meters)	443	467	
	1002	NYHA class	II	I	18 months
		BNP (pg/mL)	942	200	
		6 MWT (meters)	405	410	
	1005	NYHA class	II	I	15 months
		BNP (pg/mL)	176	44	
		6 MWT (meters)	427	435	
Adult - High Dose	1006	NYHA class	II	I	12 months
		BNP (pg/mL)	123	41	
		6 MWT (meters)	436	492	

* Corticosteroid compliance not closely monitored in initial patient
NYHA = New York Heart Association

BNP = Brain Natriuretic Peptide
6MWT = 6-Minute Walk Test

rocket pharma

RP-A501: Endomyocardial LAMP2B Protein Expression by Immunohistochemistry (IHC) and Western Blot

Cohort	Patient ID	LAMP2B Protein Expression (by IHC)**	LAMP2B Protein Expression (by Western Blot)
		Month 12	Month 5-18
Adult – Low Dose	1001*	2.5% (Previously <15%)[1]	17.9%[4]
	1002	67.8%	21.2%[5]
	1005	92.4%[2]	61.1%[6]
Adult – High Dose	1006	100%	18.2%[4]
	1007	100%[3]	RV: 45.1%[7] LV: 44.0%[7]

[1] Previously disclosed as a range due to high variance, now clarified
[2] Month 9 data
[3] Explant sample at Month 5
[4] Month 6 data; inadequate sample at Month 12
[5] Month 18 data; inadequate sample at Month 12
[6] Month 9 data
[7] Explanted heart; Month 5 data

* Patient 1001 was only locally monitored for compliance for two weeks; longer compliance monitoring initiated after 1001
** 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. Qualitative assessment reported for samples with high variance.

rocket pharma

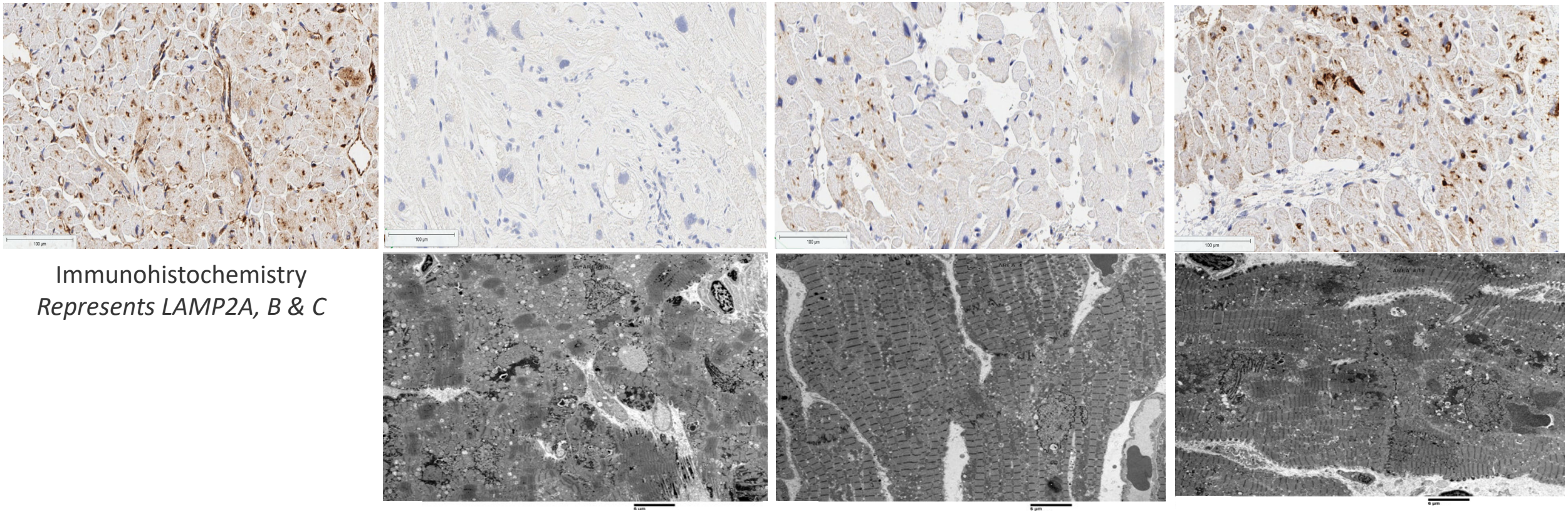
RP-A501 Low Dose: LAMP2 Protein Expression by Immunohistochemistry and Cell Morphology by Electron Microscopy

Normal Control

Baseline

Week 8

Month 9



Immunohistochemistry
Represents LAMP2A, B & C

Electron Microscopy

Representative images from patient 1005 from biopsy of IVS

Danon Hypertrophic Cardiomyopathy Physiology and Endpoints

Increased Wall Thickness

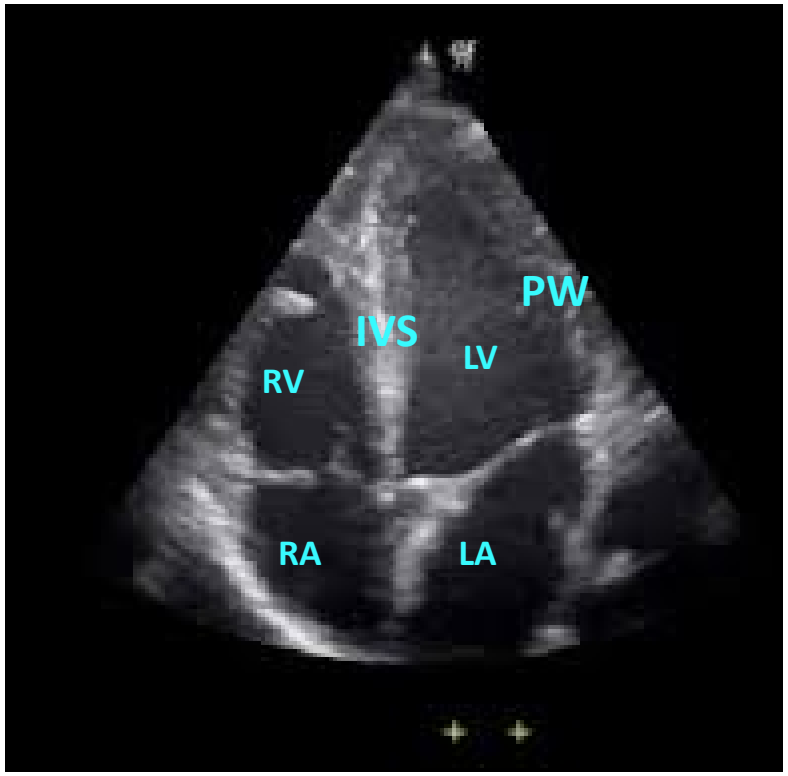
↑ LV posterior and septal wall thickness (Echo, cMRI)

Decreased Diastolic Relaxation

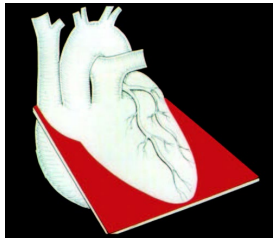
- ↑ Diastolic filling pressures
  - ↑ Pulmonary wedge pressure (invasive hemodynamics at R heart cath)
- Preserved left ventricular ejection fraction [%] (Echo, cMRI)

RP-A501: Danon Patient's Heart Thickened on Echo

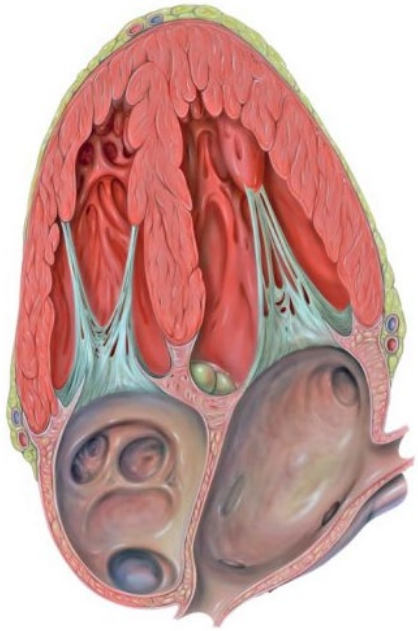
Normal



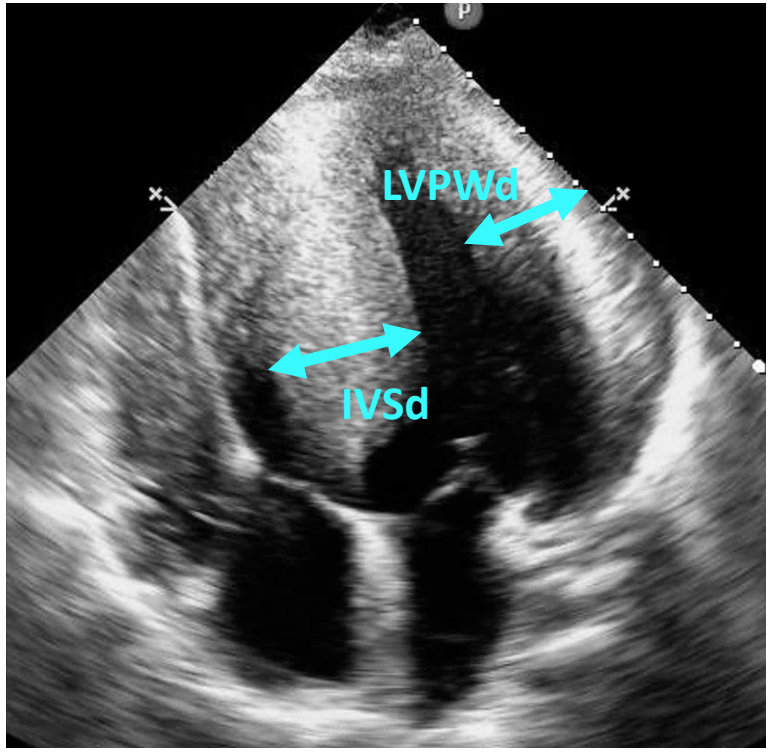
RA: right atrium
RV: right ventricle
IVS: interventricular septum
LA: left atrium
LV: left ventricle
PW: posterior wall



Apical 4-Chamber View



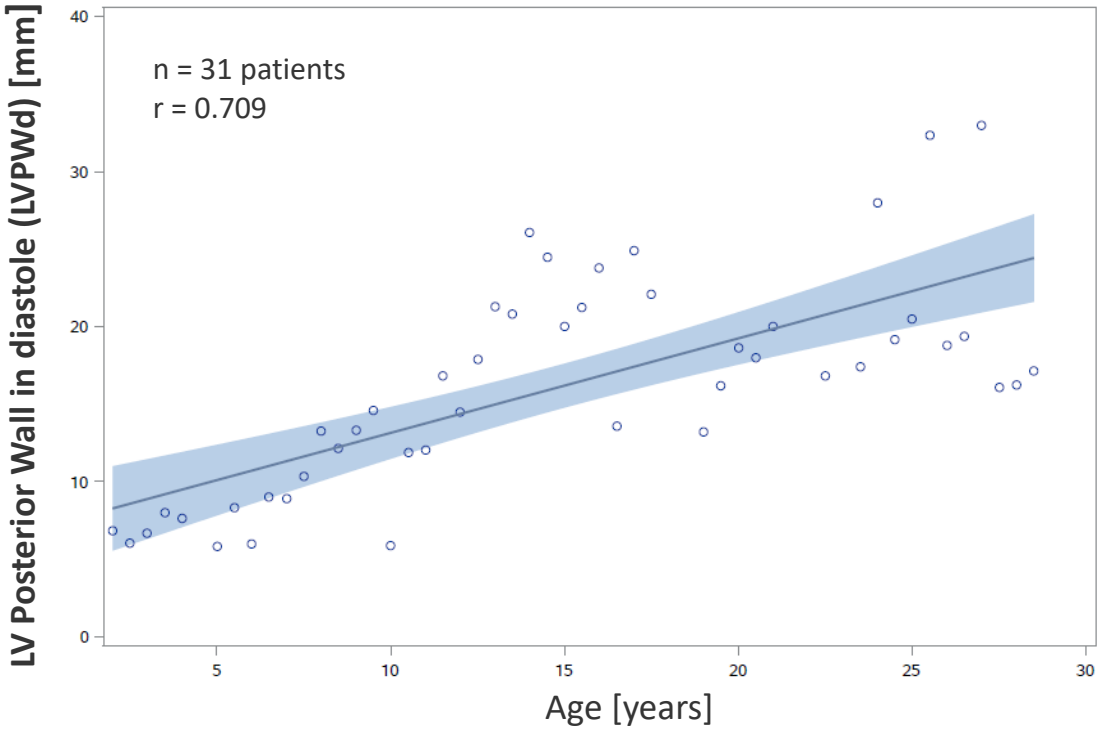
Danon Patient 1002



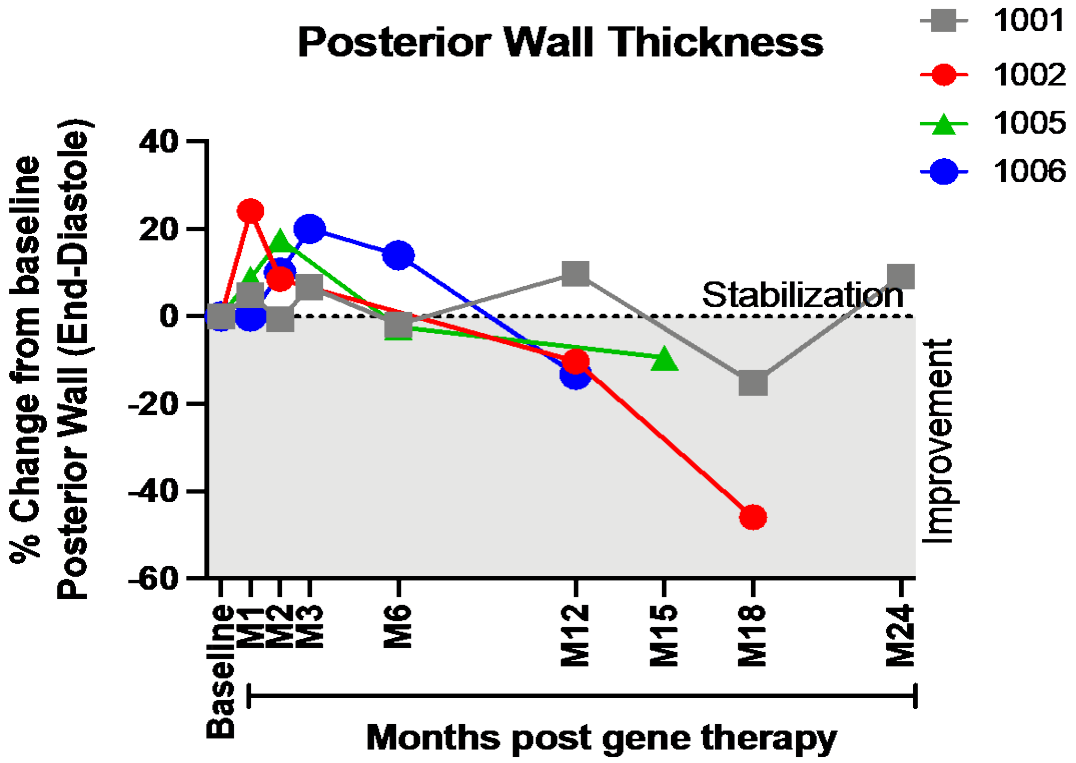
LVPWd: LVPW in diastole
IVSd: IVS in diastole

RP-A501: Treated Patients* Show Trend Toward Improvements in LV Wall Thickness vs. Untreated** Danon Males

Untreated Danon Males



RP-A501 Treated Patients



LV posterior wall thickens by 0.74 ± 0.12 mm/year in untreated Danon males**

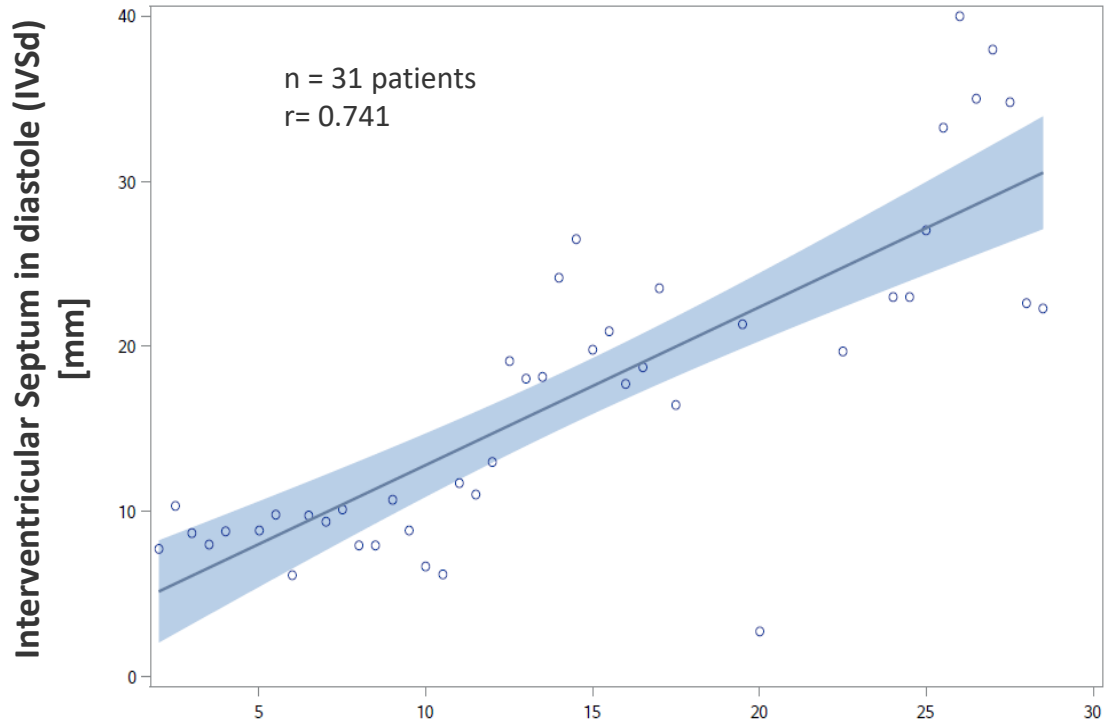
* All echocardiographic parameters from local laboratory assessment; Posterior wall: LVPWd, Septal wall: IVS

** Unpublished data from International Danon Disease Registry

*** Sutton et al. *Br Heart J. 1982; 48: 342-51*
Sluysman & Colan, 2009. Echo. in Ped. & Congenital Heart Disease

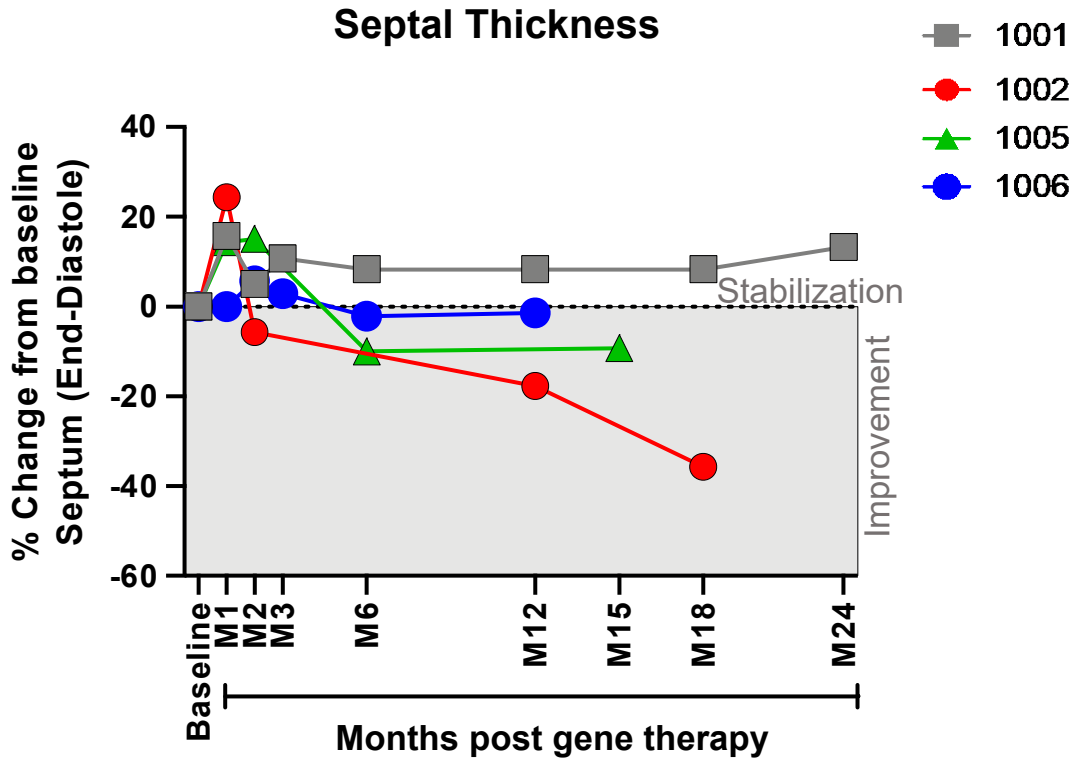
RP-A501: Treated Patients* Show Trend Toward Improvements in Septal Wall Thickness vs. Untreated** Danon Males

Untreated Danon Males



Septal wall thickens by 0.92 ± 0.15 mm/year in untreated Danon males*

RP-A501 Treated Patients



* All echocardiographic parameters from local laboratory assessment; Posterior wall: LVPWd, Septal wall: IVS

** Unpublished data from International Danon Disease Registry

*** Sutton et al. *Br Heart J. 1982; 48: 342-51*
Sluysman & Colan, 2009. Echo. in Ped. & Congenital Heart Disease

rocket pharma

Danon-related Increases in Left Ventricular Posterior Wall Corroborated by Longitudinal Echo Results in Younger Patients *

- Male Danon disease patients evaluated over median 3.1 years (range: ~ 2.4 – 12.2 years)
- Age at initial evaluation median 8.6y (LVPWd) (range: ~ 5.7– 12.0 years)

Echo Parameter	N	Mean Rate of Increase (mm/year)	Median Rate of Increase (mm/year)
LVPWd	10	1.42	1.20

Rates of wall thickening identified on larger population-based analyses (previous slide) are likely to be comparable or increased when studied in individual patients, including patients in the 8-14 y age range.

- All echocardiographic parameters from local laboratory assessment; Posterior wall: LVPW
  Unpublished data from International Danon Disease Registry
  Rates estimated via interpolation

RP-A501: Gene Therapy Has Potential to Alter Natural History of Danon Disease Patients

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

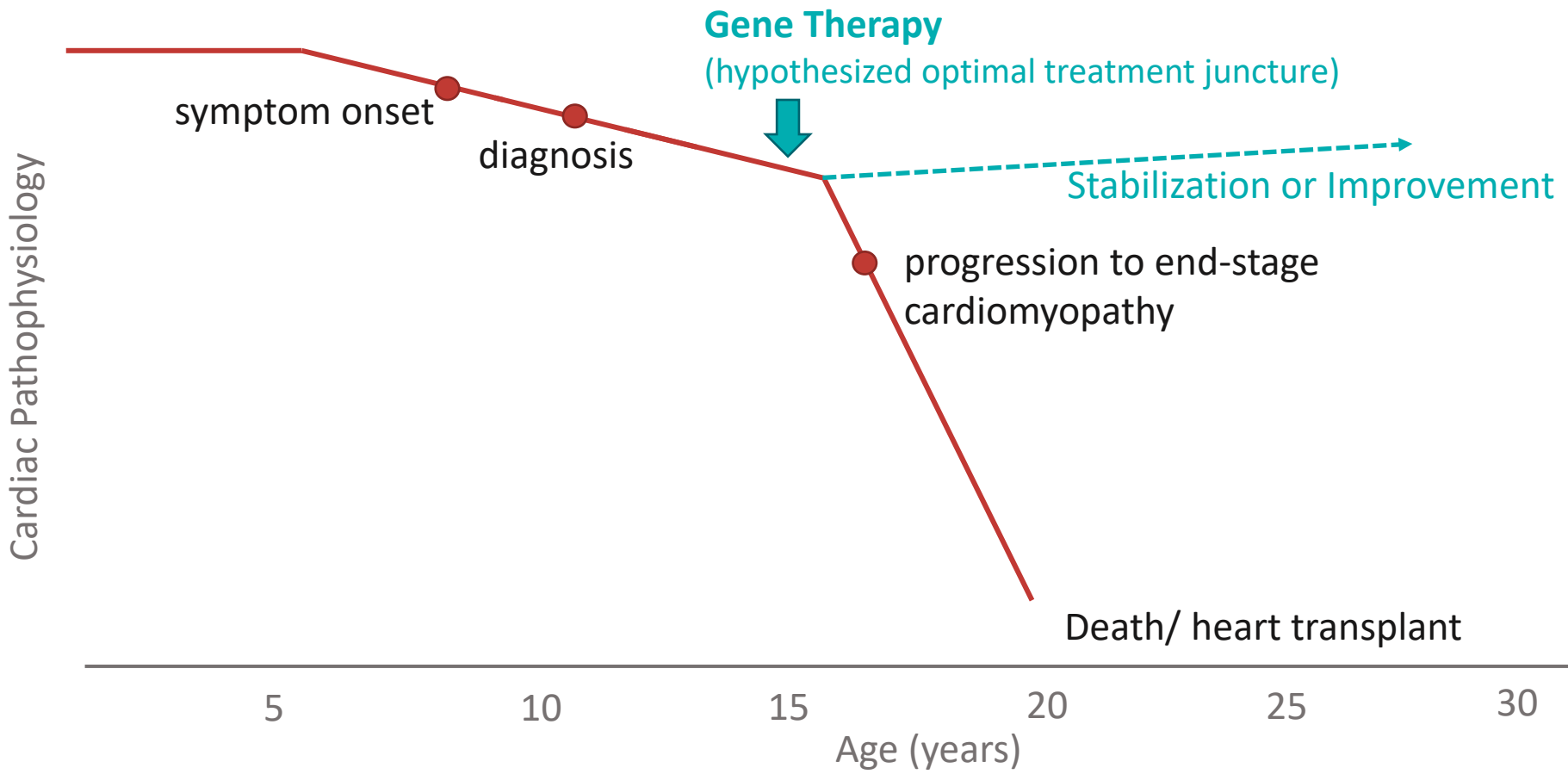


Figure modified from disease progression by age data in Boucek et al. Natural history of Danon disease. Genet Med. 2011 Jun;13(6):563-8, Brambatti et al. J Cardiac Failure. 2019. Aug;25(8):1-7, and Feingold et al. Am J Transplant. 2015 Nov;15(11):2978-85

RP-A501: Overview of Development Plan

Current Status

- ✓ Adult High- and Low-dose cohorts completed
- ✓ First patient dosed in pediatric cohort
- ✓ Orphan Drug, Rare Pediatric & Fast Track designations in the US (Eligible for PRV)

Planned Next Steps

- ❑ Completion of pediatric cohort dosing and follow-up
- ❑ End of Phase I Regulatory meeting with FDA
- ❑ Initiation of European study sites (for Phase II)
- ❑ Expanded natural history with additional longitudinal patient data in EU

Additional Life-cycle Management Activities:

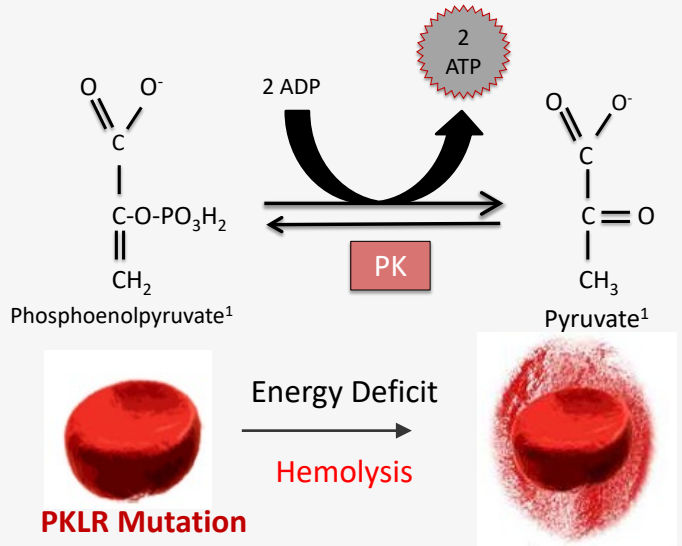
- Potential label expansion to include female Danon patients

Planned US & EU Registrational Phase II Study

Pyruvate Kinase Deficiency (PKD):
A Monogenic Red Blood Cell Hemolytic Disorder

RP-A501	**RP-L301**	RP-L102	RP-L201
Danon Disease	**Pyruvate Kinase Deficiency**	Fanconi Anemia	Leukocyte Adhesion Deficiency-I

MECHANISM OF ACTION:



Current Available Treatments: ***Chronic*** blood transfusions and splenectomy—side effects include iron overload and extensive ***end-organ damage***

Addressable Market[2]: ***~250-500*** patients/year for transfusion-dependent post-splenectomy patients

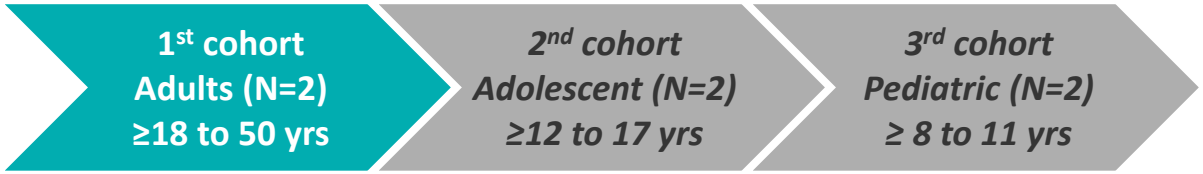
- Conservative estimates indicate a total number from ***3,000*** to ***8,000*** in the US, Europe and RoW combined
- Largest LV program in Rocket's Wave 1 pipeline

[1]One glucose molecule is metabolized into two Phosphoenolpyruvate and ultimately two Pyruvate (pyruvic acid) molecules; this final enzymatic step yields two additional ATPs from each glucose molecule

RP-L301 (PKD): Phase 1 Overview of Clinical Study Design

- Patients with ***prior splenectomy*** and ***severe and/or transfusion-dependent anemia***
- Primary Endpoint: Safety and toxicity of RP-L301
- Secondary Endpoints:
  - Clinically significant reduction of anemia
  - ***Transfusion independence*** at 12- months
  - 50% reduction in transfusion requirements
  - ***PB and BM*** genetic correction (VCN)
  - Reduction of hemolysis

Phase I Cohort Dosing Plan



✓ **Completed:** N=2 enrolled with >12M follow-up

➢ **In communication with Agencies to combine 2nd and 3rd cohorts to ≥8 to 17 yrs (N=2-3)**

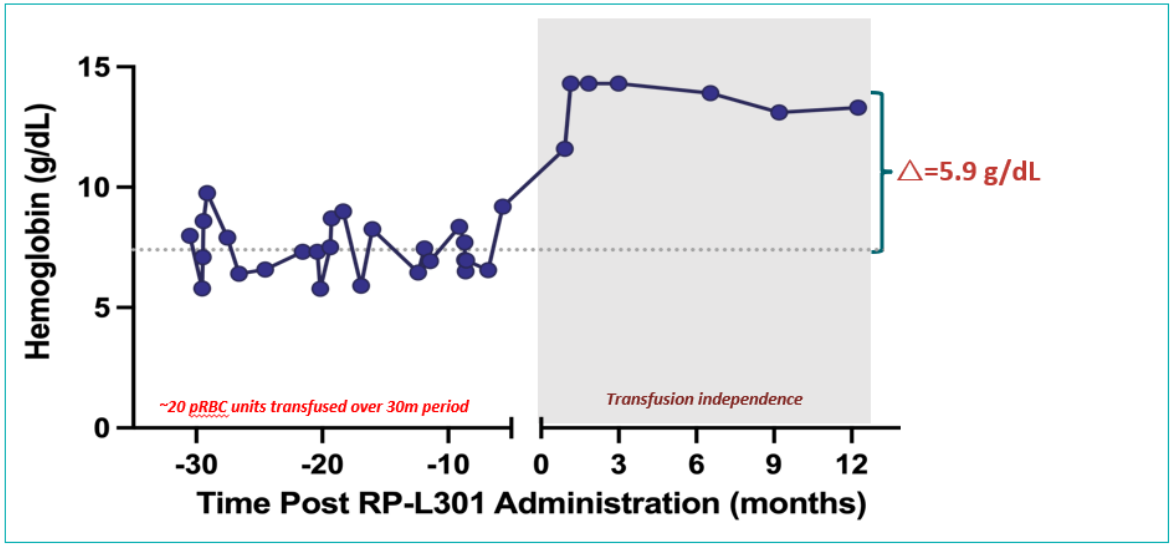
Pediatric Cohort* (N=2-3) ≥ 8 to 17 yrs

❑ Dosing likely to be initiated in **Q2** pending Agency alignment

*Discussions ongoing w/ Agency

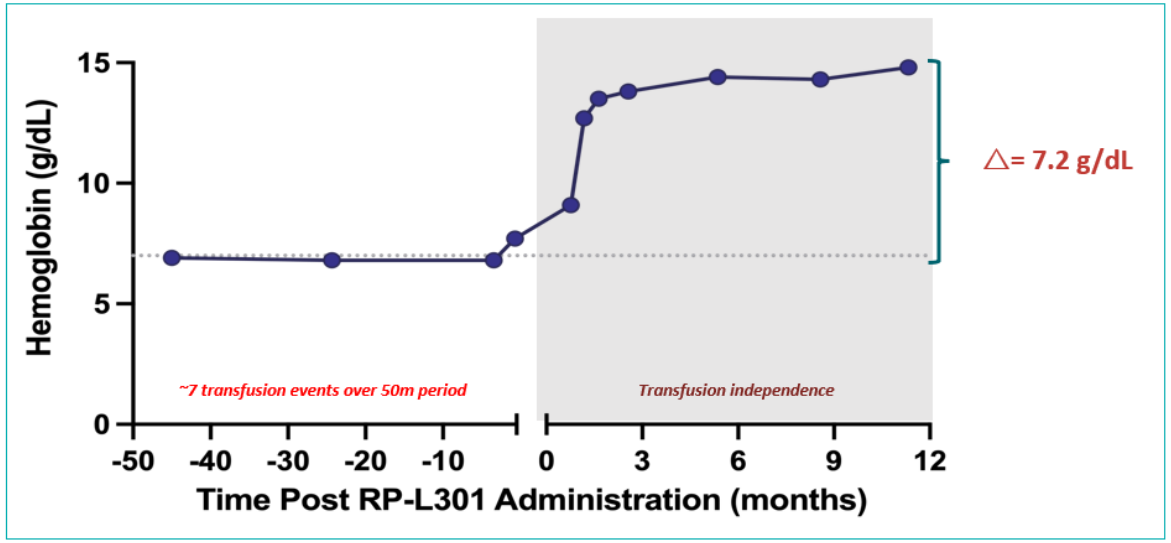
RP-L301 (PKD): Patients 1001 & 1002 Efficacy Results at 1 Year

Patient 1001



- Marked hemoglobin improvement (~7.4 g/dL to 13.3 g/dL) sustained at 12 months post-infusion
- No transfusion requirements following engraftment

Patient 1002



- Hemoglobin normalized to 14.8 g/dL at ~12 months post-infusion
- No transfusion requirements following engraftment

Accompanied by sustained pVCN in 1-3 range and improvement of hemolysis markers

Note: Lab Values during mobilization/apheresis & post-conditioning period were not included

Data as of December 2021

RP-L301 (PKD) Conclusion: Sustained Safety and Efficacy Observed in First 2 Patients at 1 Year

- Safety profile of RP-L301 ***appears favorable*** with no IP-related SAEs
- Preliminary efficacy sustained to date
  - Normalized Hgb and improving hemolysis markers
  - No red blood cell transfusion requirements post-engraftment
- Commercial-grade drug product and centralized testing for all treated patients

RP-L301 (PKD): Development Plan

PKD Study Progress to Phase II and Launch

- ✓ Key Endpoints selected
  - Hgb increase
  - ↓50% transfusions or transfusion independence
- ✓ Well delineated natural history in recent PKD NHS publications
- ❑ Complete Phase I pediatric cohort dosing (N=2-3)
- ❑ End of Phase I Regulatory meeting with FDA
- ❑ Approve & Launch RP-L301 seek regulatory approval in the U.S. and EU

Life-Cycle Management

- ❑ Expansion study to pre-splenectomy patients anticipated in 2023
- ❑ Exploration of non-genotoxic conditioning

rocket pharma

Fanconi Anemia (FA): *Monogenic DNA-repair disorder*

RP-A501
Danon Disease

RP-L301
Pyruvate Kinase Deficiency

RP-L102
Fanconi Anemia

RP-L201
Leukocyte Adhesion Deficiency-I

Background: 80% of patients experience BMF within the ***1st decade of life.*** Patients have a predisposition to ***hematologic malignancies*** and solid tumors

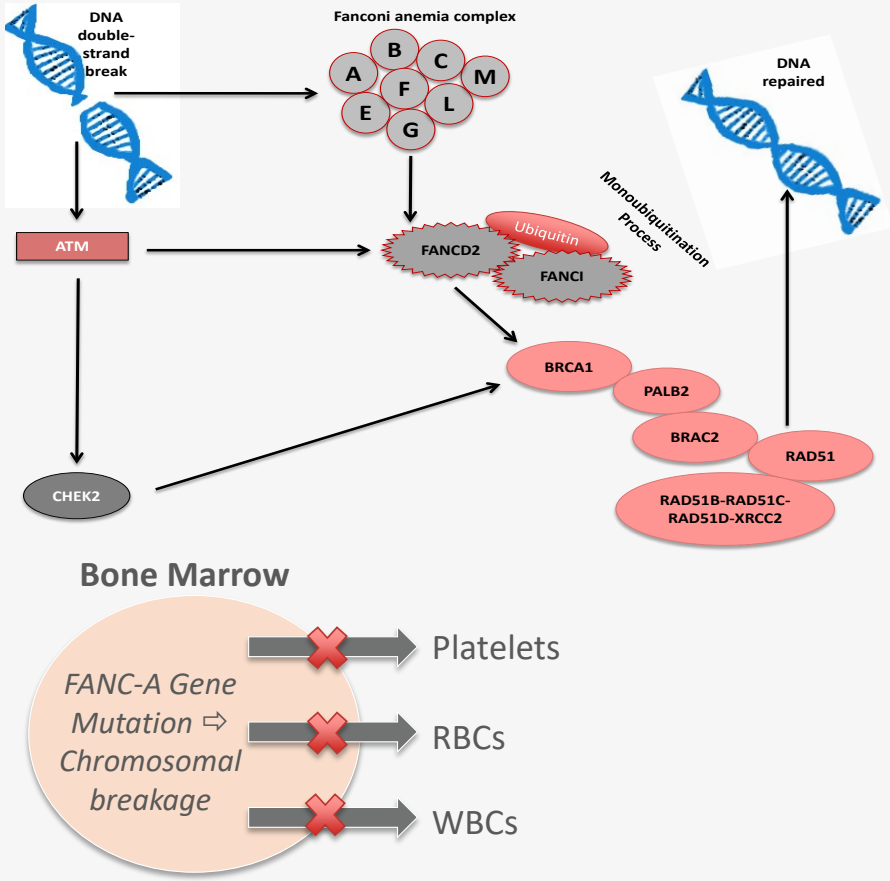
- Acute Myeloid Leukemia
- Head and Neck Cancer[1]

} ↑ Risk 30-50x

Current available treatments: Allogeneic hematopoietic stem cell transplant associated with 100-day mortality, GVHD, and additional increased cancer risk

Addressable Market[2]: Estimated US + Europe ~***4000*** *patients (prevalence),* ***500 patients/year***

MECHANISM OF ACTION



[1] Alter Br J Hametol 2010.
[2] 4,000 based on a detailed population analysis of FA genomic variants. 500 per year extrapolated by actual transplants per year plus patients from prevalence

RP-L102 (FA): Clinical Program Overview

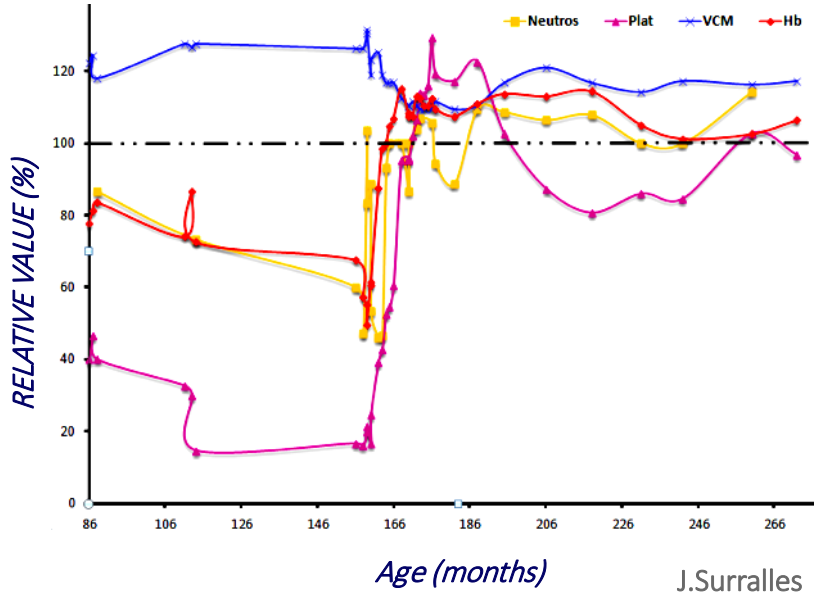
RP-L102 Studies	Non-randomized, open label studies: US Phase 1, US Phase 2, and EU Phase 2 (FANCOLEN-II)	
CMC/Drug Product	**"Process B"** includes cell enrichment, transduction enhancers, commercial-grade vector and modified cell processing	
Clinical Studies	✓ EU FANCOLEN-I study (n=9) completed ✓ US Phase 1 study (n=2) completed at Stanford University ❑ US Phase 2 study ongoing at Stanford and Minnesota University ❑ EU Phase 2 study ongoing at Hospital Infantil Universitario Niño Jesús and UCL/GOSH	
Endpoints	**Efficacy**	**Engraftment**: Peripheral blood (PB) and BM vector copy number (VCN) **Phenotypic correction**: Increased resistance of BM and PB cells to MMC and DEB **Clinical response**: Prevention of BMF
	Primary Endpoint: ***Efficacy in at least patients (defined as >10% increase in MMC resistance at 2 time points observed between 12-36M post-rx) required to reject null hypothesis***	
	Safety profile of RP-L102	

Post rx = Post-treatment

RP-L102 (FA): Potential to Correct Bone Marrow Defect Without Conditioning to Prevent Hematologic Failure

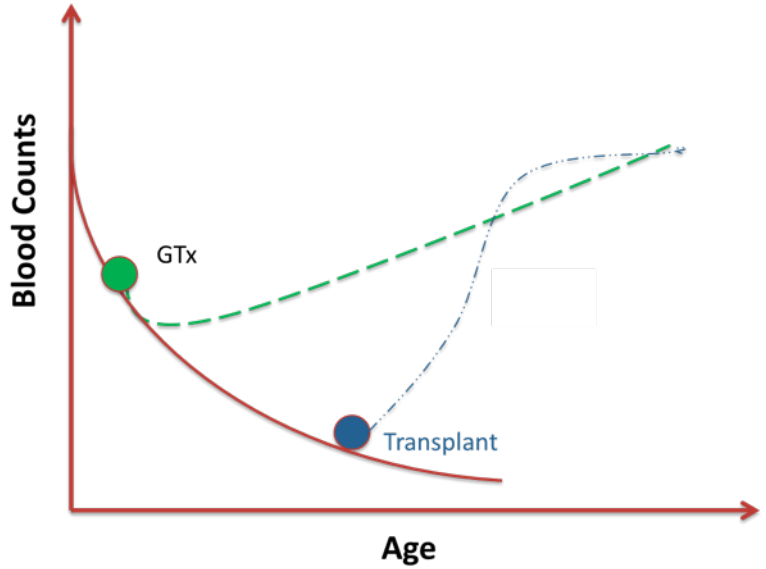
Rationale for GTx in FA Based on Somatic Mosaicism

- Small proportion of FA patients ***spontaneously revert*** to normal phenotype
- ***Proliferative advantage*** favors gene-corrected cells



Gene Therapy Value Proposition:

- Potential to ***correct*** blood & bone marrow defect ***without conditioning***
- GTx implemented as preventative measure to ***avert bone marrow failure***



Potential for single-corrected cell to repopulate to healthier bone marrow[1,2]

[1] Soulier, J., et al. (2005) Detection of somatic mosaicism and classification of Fanconi anemia patients by analysis of the FA/BRCA pathway. *Blood* 105: 1329-1336; [2]Data on file: Showing a single patient with a spontaneous correction of blood counts, no therapy administered.

RP-L102 (FA): Highly Favorable Safety and Initial Efficacy Observed

Safety Data

Safety results appear *highly favorable* and RP-L102 well positioned as *potential first-line treatment option

- Patients treated **without conditioning**
- No signs of dysplasia
- Therapy does not preclude later allogeneic transplant

Efficacy Data

11 patients received RP-L102
7 of 9 show preliminary evidence of engraftment

N = 8 with ≥ 12M follow-up (12-32M)

To date, **6 of 8** show increasing evidence of engraftment with BM MMC resistance ranging from **16-63%** (at least one timepoint)

N = 3 with < 12M follow-up

Longer term data to follow

*In CIEMAT Previous FANCOLEN-I trial RP-L102 related SAEs: 1 transient infusion-related reaction (Grade 2)

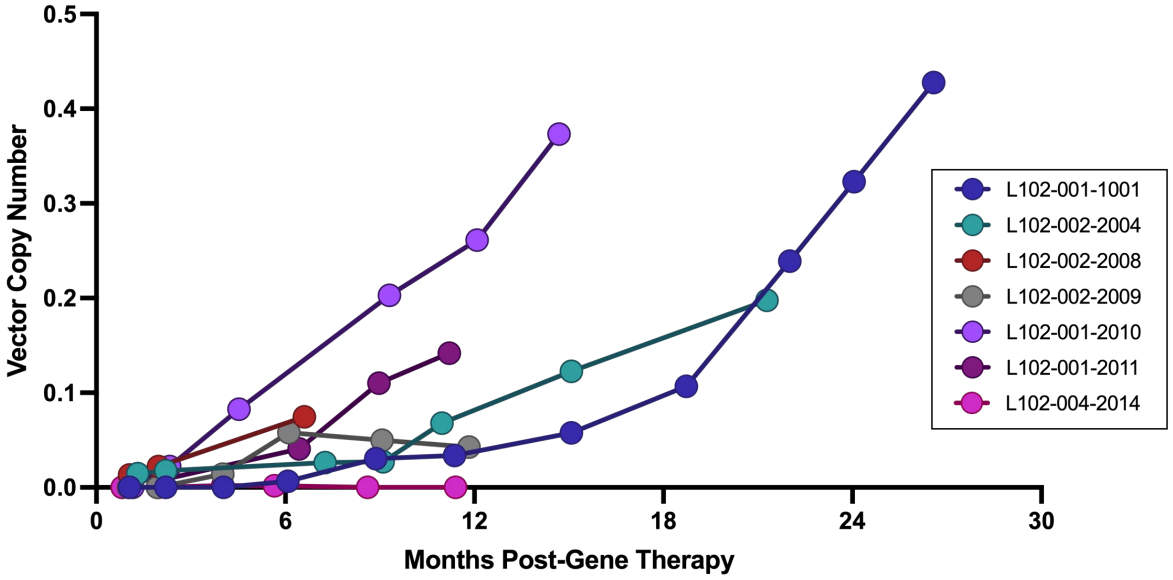
RP-L102 (FA): Treated Patients

Phase	Subject #	Site	Age at Enrollment	Gender	Key Drug Product Characteristics		Follow-up
					CD34+ Cells/kg	Mean VCN: Liquid Culture	
PH 1	1 (1001)	US	5	F	2.0×10^5	2.08	32M
	2 (1002)*	US	6	F	3.7×10^5	2.21	18M*
PH 2	3 (2004)	Spain	3	M	4.8×10^5	1.70	21M
	4 (2008)	Spain	2	F	3.2×10^6	1.65	15M
	5 (2009)	Spain	3	M	1.9×10^6	2.16	15M
	6 (2010)	US	3	M	4.1×10^6	0.62	15M
	7 (2011)	US	5	F	2.8×10^6	1.46	15M
	8 (2014)	UK	6	F	5.4×10^5	3.68	12M
	9 (2016)	US	2	M	3.0×10^5	1.96	9M
	10 (2021)	UK	2	F	2.3×10^6	pending	~2M†
	11 (2023)	UK	5	F	2.5×10^5	pending	0M‡

** Subject withdrawn from the study at 18 months post-RP-L102 infusion; received successful allogeneic HSCT*
† Subject recently received RP-L102 infusion as of October 2021
‡ Subject recently received RP-L102 infusion as of December 2021

rocket pharma

RP-L102 (FA): Patients with ≥ 12M Follow-Up Demonstrate Evidence of Genetic and Phenotypic Correction



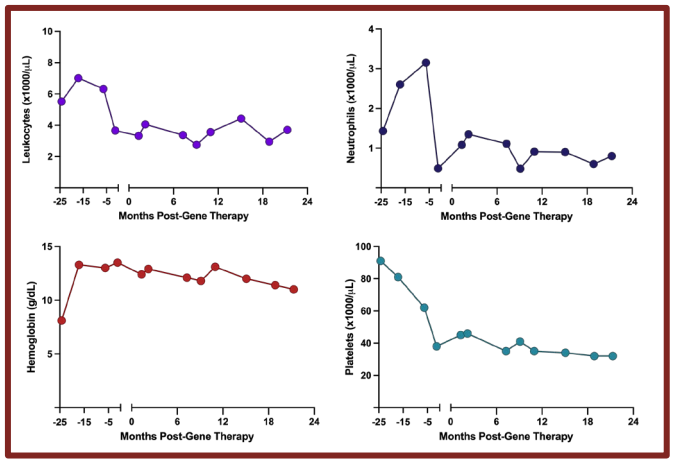
- **Sustained PB VCN in 6 of 8 patients with ≥ 12 months of follow-up**
- **Concomitant BM CFC MMC resistance ≥ 10% above baseline values in 6 of 8 patients**

Subject #	Patient Age at Treatment	Bone Marrow Assessment Performed (months)	BM CFC MMC Resistance at 10 nM MMC (%)
1 (1001)	5	24	16†
3 (2004)	3	21	63
4 (2008)	2	12	21
5 (2009)	3	12	29
6 (2010)	3	12	42
7 (2011)	5	12	31
8 (2014)	6	12	0

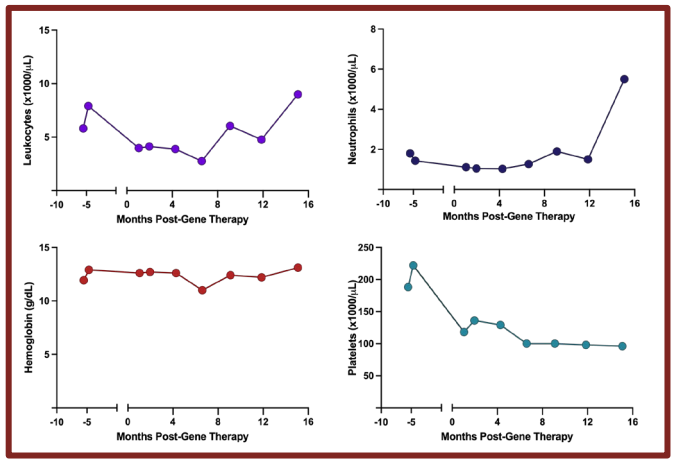
† Assessment was not performed at study's centralized laboratories

RP-L102 (FA): Blood Count Stabilization in At Least 5 Patients

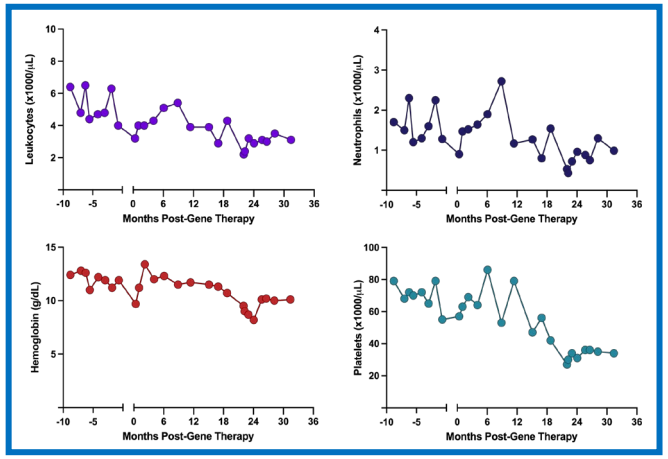
Subject 3 (2004)



Subject 4 (2008)

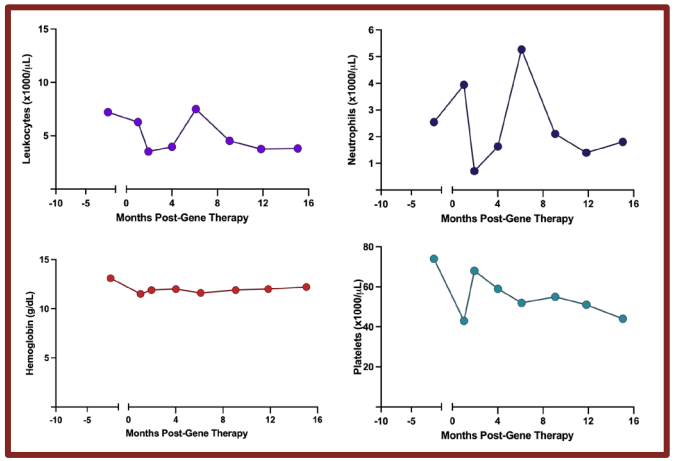


Subject 1 (1001)

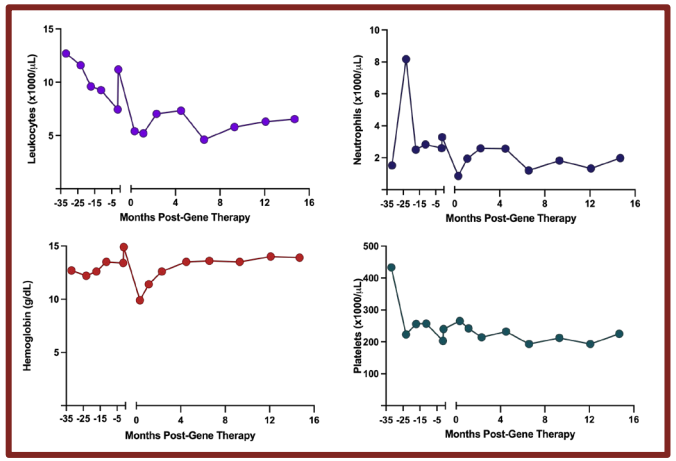


- **Subject 1 (1001; BM CFC MMC Resistance 16%) had modest decline in blood counts with potential stabilization after ~18m; no transfusions have been required**

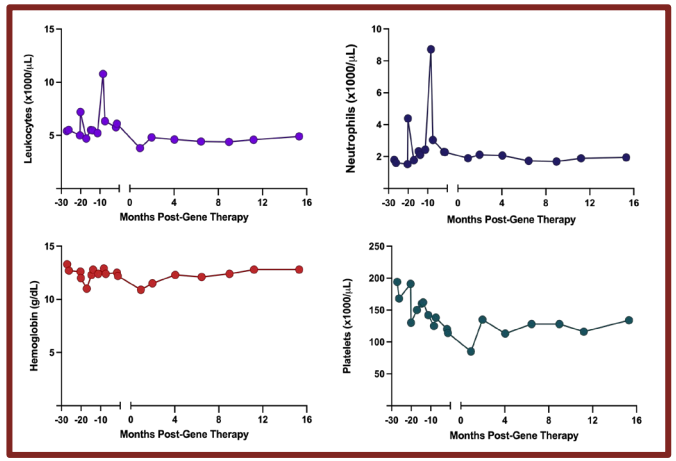
Subject 5 (2009)



Subject 6 (2010)



Subject 7 (2011)



RP-L102 (FA): Path to Product Registration

- ✓ **Initial Efficacy and Highly Favorable Safety Profile:**
  - ➢ Initial efficacy in 6 of 8 patients with >12M follow-up
  - ➢ No cytotoxic conditioning used, only one transient infusion-related SAE (Grade 2)
- ✓ **Regulatory Designations:**
  - ➢ Orphan Drug designation in the US/EU
  - ➢ **Rare Pediatric Disease designation (eligible for PRV)**
  - ➢ Fast Track (US), Advanced Therapy Medicinal Product (ATMP)
  - ➢ **Regenerative Medicine Advanced Therapy (RMAT), PRIority MEdicines (PRIME)**
- ❑ **Topline Data Readout Anticipated 3Q-2022**
  - ➢ Rejection of null hypothesis with minimum of 5 patients with increased MMC resistance >10% at **two timepoints** between 12-36M

Additional Life-cycle Management Activities:

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

Anticipated Simultaneous
BLA / MAA Filing

rocket pharma

Leukocyte Adhesion Deficiency-I (LAD-I): A *Monogenic Immunodeficiency*

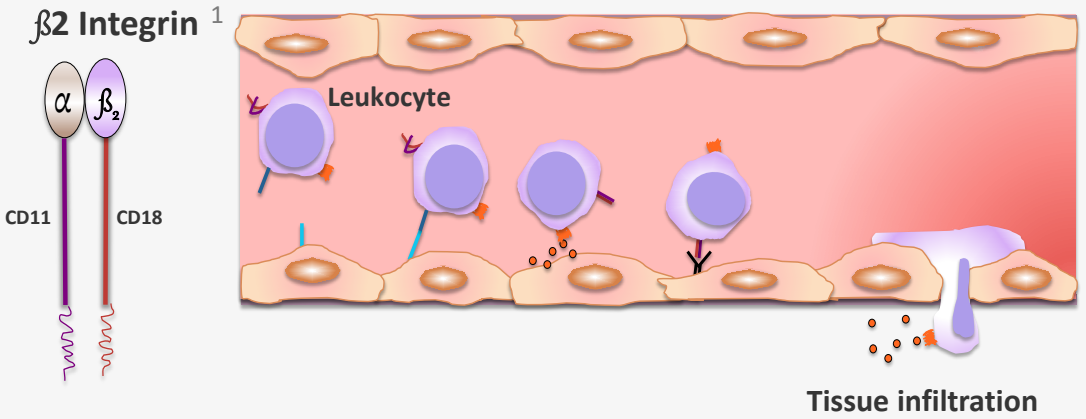
RP-A501
Danon Disease

RP-L301
Pyruvate Kinase Deficiency

RP-L102
Fanconi Anemia

RP-L201
Leukocyte Adhesion Deficiency-I

MECHANISM OF ACTION:



Background: Recurring and ultimately fatal infections caused by *ITGB2* gene mutations; ***>50%*** patients with severe variant: ***60-75% mortality by age 2***

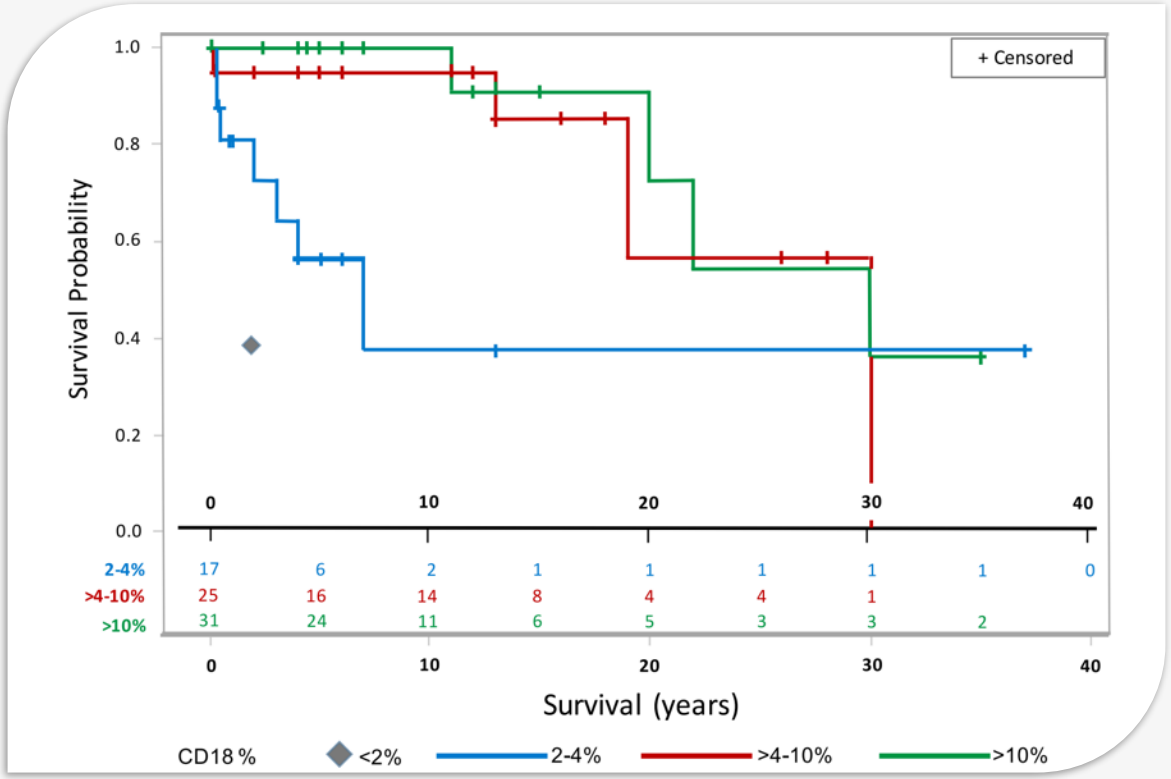
Current Available Treatments: Allogeneic hematopoietic stem cell transplant associated with significant graft failure and acute GVHD

Addressable Market: Estimated ***25-50 pts*** treatable per year for severe population; ***up to 100*** for potential expansion into moderate population

[1] Defective expression of ß2 integrin on leukocytes limits their extravasation to inflamed sites.

Rationale for Gene Therapy in LAD-I: CD18 Expression Correlates to Patient Survival

Kaplan-Meier Survival Estimates by Neutrophil CD18 Expression
-Patients with moderate LAD-I not receiving allogeneic HSCT-



Natural history studies show the ***correlation*** between ***higher CD18*** expression and longer patient ***survival,*** supporting gene therapy's potential in LAD-I patients

CD18 > 10% results in survival of severe LAD-I patients for decades

The grey diamond indicates the 39% survival to age 2 years for 66 evaluable patients with severe LAD-I not receiving HSCT

Poster Presentation at ASGCT May 2018

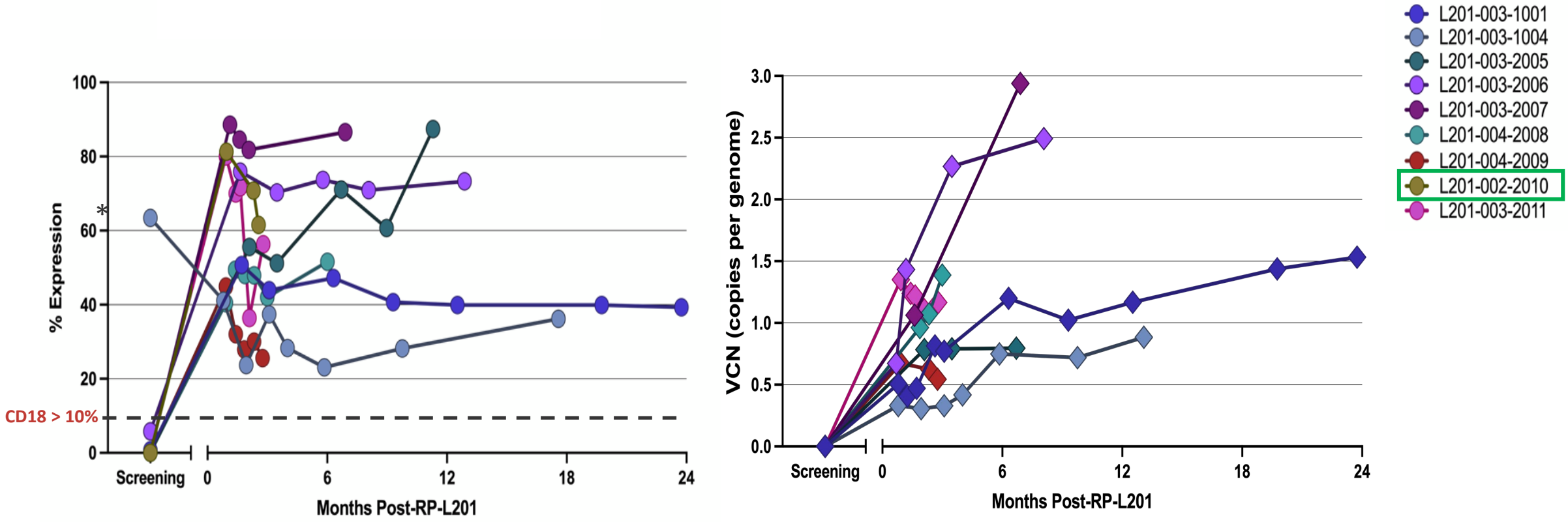
Source: Almarza Novoa E et al. J Allergy Clin Immunol Pract. 2018 Jan 20. pii: S2213-2198(17)31026-7. [Epub ahead of print]

RP-L201 (LAD-I): Subject and Cell Product Characteristics

Patient ID	Gender	Age (enrollment)	Drug Product VCN	CD34+ Cell Dose
1 (1001)	F	9 yrs.	3.8	4.2×10^6 cells/kg
2 (1004)	F	3 yrs.	2.5	2.8×10^6 cells/kg
3 (2005)	F	3 yrs.	1.8	6.5×10^6 cells/kg
4 (2006)	M	7 mo.	2.9	4.3×10^6 cells/kg
5 (2007)	M	3 mo.	3.6	5.0×10^6 cells/kg
6 (2008)	M	5 mo.	3.8	3.3×10^6 cells/kg
7 (2009)	M	3 yrs.	2.0	4.5×10^6 cells/kg
8 (2011)	F	2 yrs.	3.8	3.8×10^6 cells/kg
9 (2010)	F	4 yrs.	3.5	10.0×10^6 cells/kg

LAD has received CIRM Funding. Data as of December 2021,

RP-L201 (LAD-I): CD18 & VCN Expression in PB Neutrophils



**Dim/weak CD18 expression reported at baseline for PT 1004 in ~60% of Cells*

All 9 of 9 patients with CD18 > 10%

LAD has received CIRM Funding. Data as of December 2021

RP-L201 (LAD-I): Data Summary

- **Phase 1/2 study complete: 9 of 9 severe LAD-I patients successfully treated**
- **Safety profile of RP-L201 appears favorable:**
  - No drug product-related SAEs
- **Efficacy evident in all 9 severe LAD-I patients with follow-up of 3-24m**
  - Includes 4 patients with ≥ 12-months of follow-up; CD18+ PMN expression ranging from ~40% to ~87% at timepoints between 12-24 months
  - No LAD-I disease-related protracted hospitalizations for any of the 9 patients following RP-L201 gene therapy
- **100% engraftment and potentially curative CD18 reconstitution across all treated patients (achieved <34 days post-infusion)**

RP-L201 (LAD-I): Global Registrational Plan

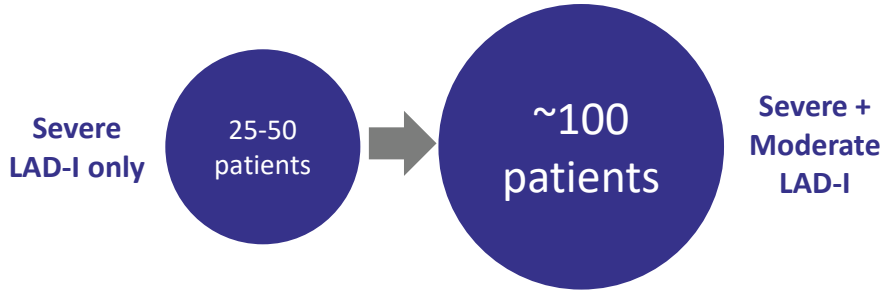
- ✓ **Enrollment Completed; 9 of 9 patients dosed**
- ✓ **Efficacy Seen in All 9 Patients with Minimum 3 Months Follow-Up**
- ✓ **Regulatory Designations:**
  - ➢ Fast Track and Advanced Therapy Medicinal Product (ATMP)
  - ➢ **Rare Pediatric Disease (eligible for PRV)**
  - ➢ Orphan Drug designation in the U.S./EU
  - ➢ **Regenerative Medicine Advanced Therapy (RMAT), PRIority MEdicines (PRIME)**
- ❑ **Topline Data Readout Anticipated 2Q-2022**
  - ➢ Survival at 12 months and to age >2 for 7 of 9 patients

BLA / MAA Filing

Life-cycle Management

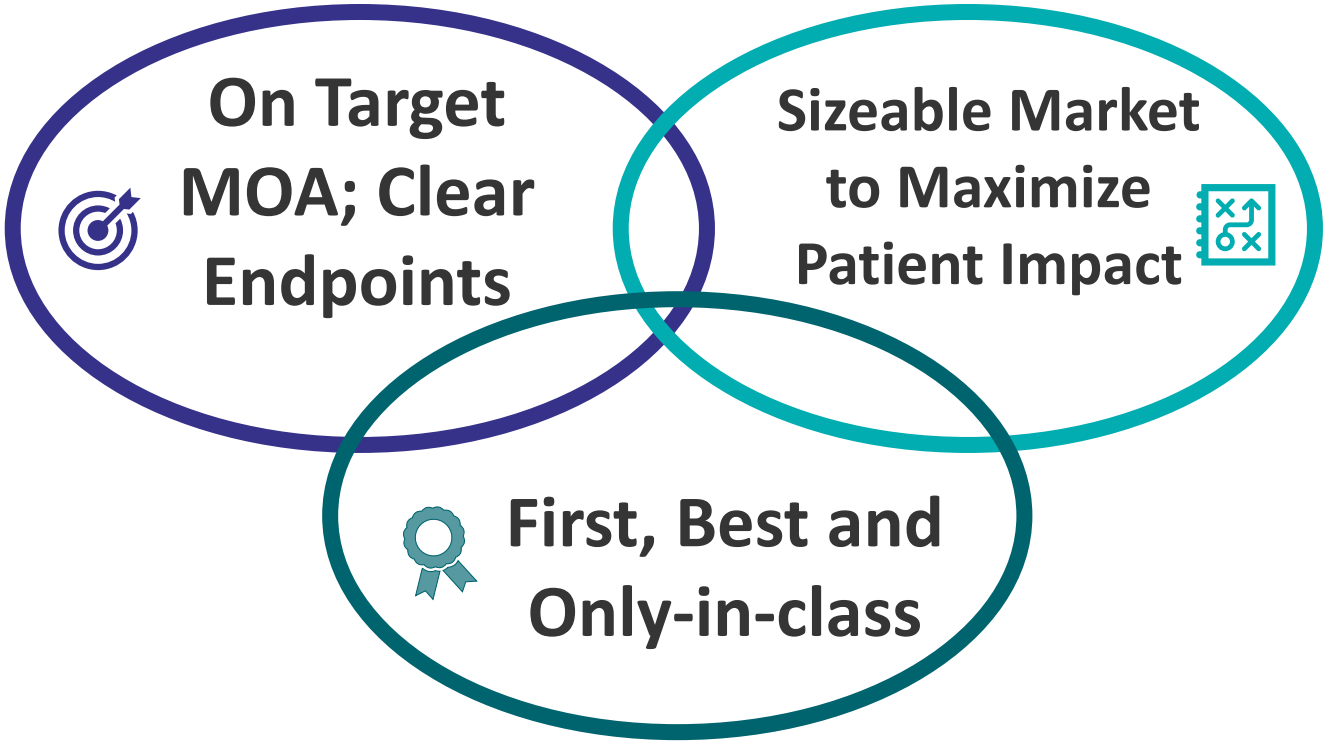
- **Potential label expansion** to include moderate LAD-I population
- Potential study initiation in 2023

LAD-I Addressable Market



Replicating Core R&D Strategy for “Wave 2” Programs

We continue to build our pipeline based on our core R&D strategy; identify the “right” indications for the most efficient development path



Anticipated Program Milestones (2022 – 2024)
Our Path from Clinical to Commercial-Stage

2022

2023-2024

2Q 2022

- **LAD-I:** Top line data
- In-house **AAV cGMP manufacturing**

3Q 2022

- **FA:** Top line data
- **Danon:** Pediatric Cohort Data

4Q 2022

- **Danon:** Initiate Phase 2 pivotal study activities
- **PKD:** Preliminary Phase 1 data readout
- **PKD:** Initiate Phase 2 pivotal study activities

- **LAD-I & FA:** Approvals & Launches in U.S. & EU
- **Wave 2 pipeline** enters clinic
- Label expansion studies for **Danon (Female), FA (C & G), LAD-I (Moderate) and PKD (Moderate)** programs

Rocket Values & Partnerships

TRUST

GENEROSITY

CURIOSITY

ELEVATE

ciber
Centro de Investigación Biomédica en Red

Ciemat

FRED HUTCH

iis FJD INSTITUTO DE INVESTIGACIÓN SANITARIA FUNDACIÓN JIMÉNEZ DÍAZ

Penn

LUND UNIVERSITY

Hospital Infantil Universitario Niño Jesús

REGENXBIO

UCL

UNIVERSITY OF MINNESOTA

UCLA

UC San Diego

Hospital Universitario Fundación Jiménez Díaz Grupo quirónsalud

Stanford Medical School