Corporate Presentation

February 2021

rocket pharma

SEEKING GENE THERAPY CURES

NASDAQ: RCKT

Important Information

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 2020 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2020, filed November 6, 2020 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.

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Mission, Vision and Values

TRUST

Trust is given and trust is earned – it's a balance. The word trust comes from the Proto-Indo-European word deru which means "to be firm, solid, steadfast." Trust is the ground and foundation for everything we do.

GENEROSITY

Being generous means following up, sharing our best ideas, forgiving ourselves and others, asking who needs us, treating our word as gold, taking time to truly see others, and so many other things. The word generous has the same root as the word "gene" – which meant "to beget." Genes thrive on the generosity of others. What more is there to say?

CURIOSITY

The wonder of a child staring up at the night sky. Humility, egoless-ness. No single one of us can do this job alone and it is ok to ask for help. Curiosity is derived from the Latin word "cura" which gave birth to the word "care" as well as "cure." Generosity is to curiosity what gene is to cure.

ELEVATE

Derived from Latin levis which means "light" as opposed to heavy. How can we bring trust, generosity and curiosity to elevate ourselves, each other, the pipeline and ultimately the life experience of patients and their families?

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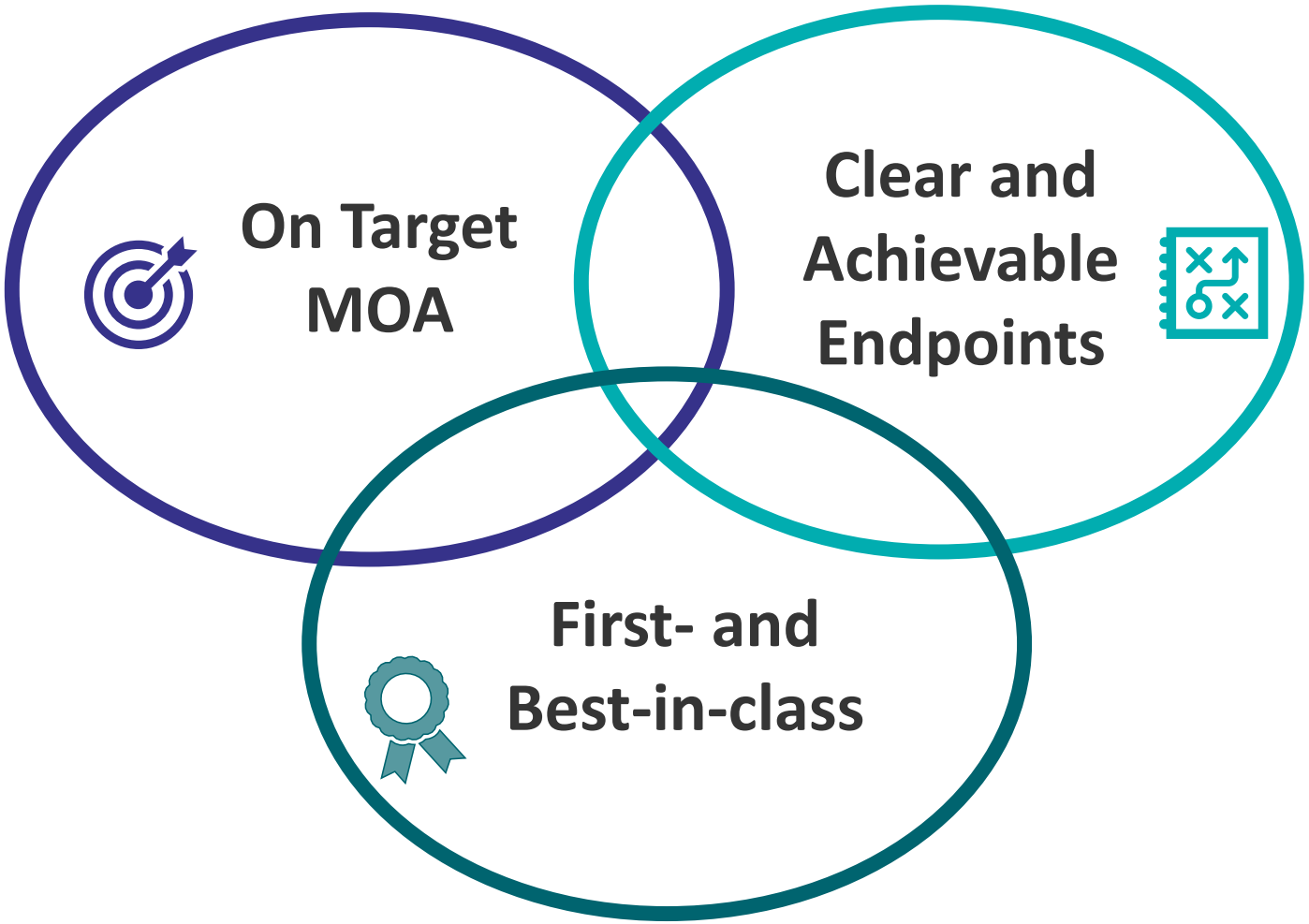
Multi-Platform Gene Therapy Targeting Rare Diseases



Focused on R&D Strategy for Sustainable Innovation

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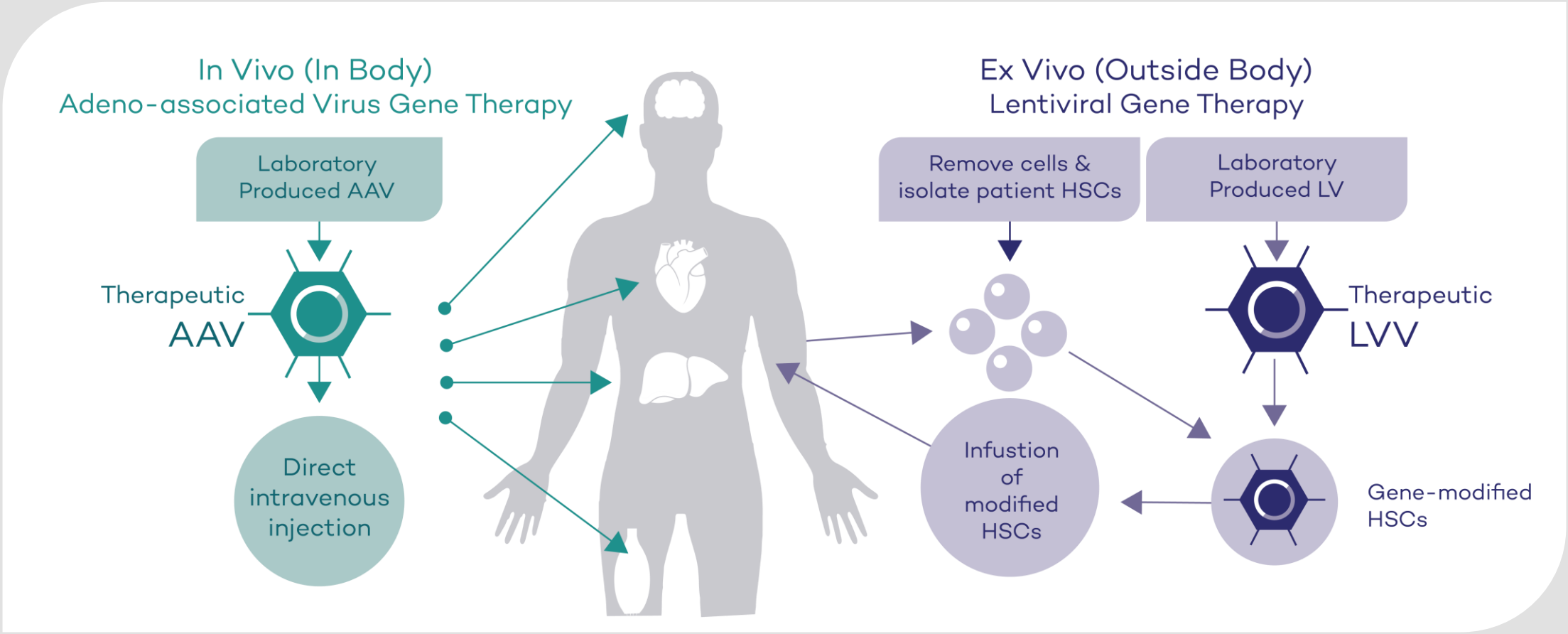
Gene Therapy: A Multi-Platform Approach



About Rocket Pharma

Multi-Platform Gene Therapy Company Targeting Rare Diseases: 1st-in-class with direct on-target mechanism of action and clearly-defined clinical endpoints

Ex-vivo Lentiviral vectors (LVV)

- Fanconi Anemia (FA)
- Leukocyte Adhesion Deficiency-I (LAD-I)
- Pyruvate Kinase Deficiency (PKD)
- Infantile Malignant Osteopetrosis (IMO)

In-vivo adeno-associated virus (AAV)

- Danon Disease

Multiple Near- & Medium-term Company Value Drivers

Near-term Milestones

- All five programs in the clinic (initiation of IMO)
- New preliminary data in Danon & PKD; Additional mature data in FA & LAD-I
- Two programs in registration-enabling Phase 2 (FA, LAD-I)

Medium-term Milestones

- First global submission (BLA)
- Platform establishment and pipeline expansion
- Current programs eligible for Pediatric Priority Review Vouchers

Strong Precedents and World-Class Expertise

Strong Precedents and Sound Strategy

- Compelling clinical proof-of-concept for LVV- & AAV-based therapies across a spectrum of genetic disorders
- Clearly-defined product metrics across indications
- Experienced company leadership
- Leading research and manufacturing partners

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Rocket's Leadership Team

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

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

Jonathan Schwartz, M.D.
CMO & Clinical Development, SVP
Led multiple biologics approvals

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

Carlos Garcia-Parada, MBA
Chief Financial Officer
14 years of Oncology & Rare Disease experience
Leading role in launching Kymriah, the first CAR-T product on the market.

José Trevejo
Chief Development Officer of AAV, SVP
~20 years of clinical development expertise

Claudine Prowse, Ph.D.
SVP, Strategy & Corporate Dev
~20 years capital markets, strategy, corporate development

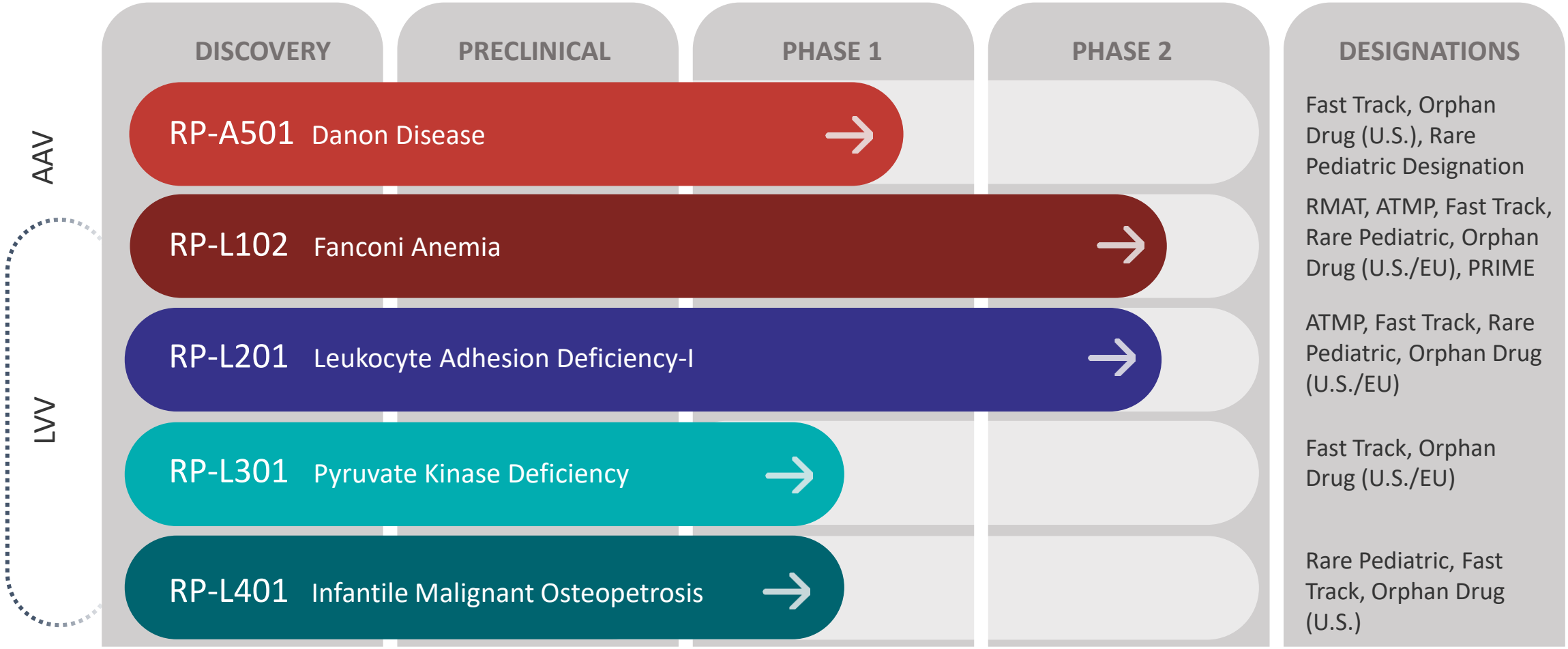
John Militello, CPA
VP, Principal Accounting Officer
~20 years public company finance and accounting experience, 6 years biotech experience

Gayatri R. Rao, M.D., J.D.
VP, Global Program Head, LVV
7-Year Former Director of FDA's Office of Orphan Products Development

Ramji Krishnan, Ph.D.
VP, Manufacturing & Manufacturing Sciences
17+ years of CMC product development and life cycle management expertise

Brian C. Beard, Ph.D.
AVP, CMC
15+ years cell and gene therapies expertise

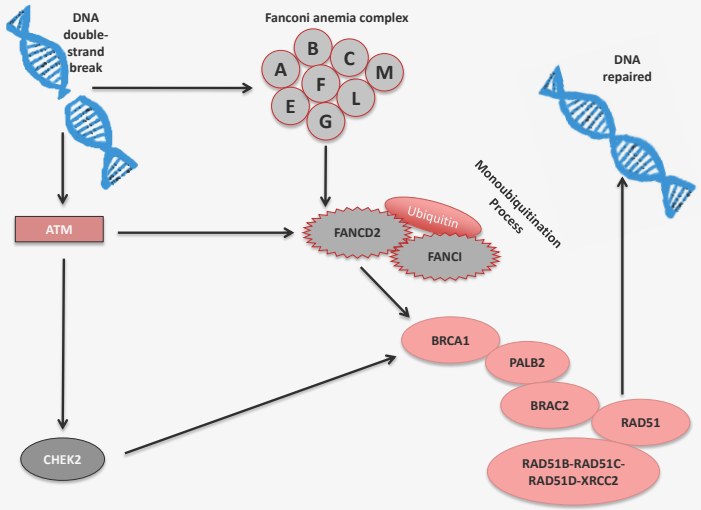
Rocket's Expanding Pipeline: Potential for Significant Value Creation Near and Long Term



Fanconi Anemia (FA)
Monogenic DNA-repair disorder

RP-L102 Fanconi Anemia | RP-A501 Danon Disease | RP-L201 Leukocyte Adhesion Deficiency-I | RP-L301 Pyruvate Kinase Deficiency | RP-L401 Infantile Malignant Osteopetrosis

OVERVIEW:



Bone Marrow

FANC-A Gene Mutation ⇨ Chromosomal breakage

- Platelets
- RBCs
- WBCs

Disease Sequelae:
- Birth Defects
- Skin Discoloration
- Developmental Issues

80% Bone Marrow Failure by Age 10

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 target population of approximately 4,000 patients, 500 patients/year

RP-L102: LVV gene therapy that elicits phenotypic correction of blood cells and stabilization of previously declining blood counts

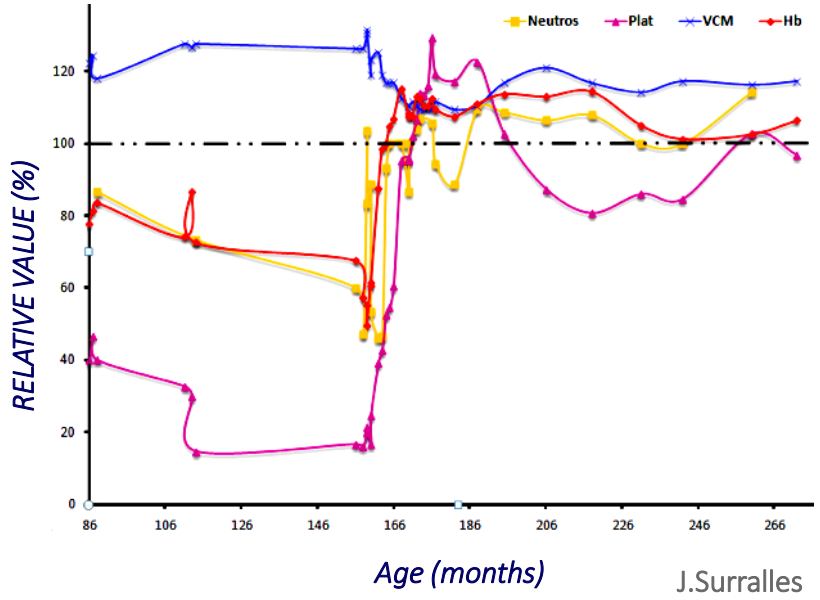
Regulatory Designations: Fast Track, Regenerative Medicine Advanced Therapy (RMAT) and Rare Pediatric Disease designations in the US; Advanced Therapy Medicinal Product (ATMP) classification and PRIority MEdicines (PRIME) in the EU; Orphan Drug designation in the US/EU

[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

Potential to Correct Bone Marrow Defect without Conditioning to Prevent Hematologic Failure

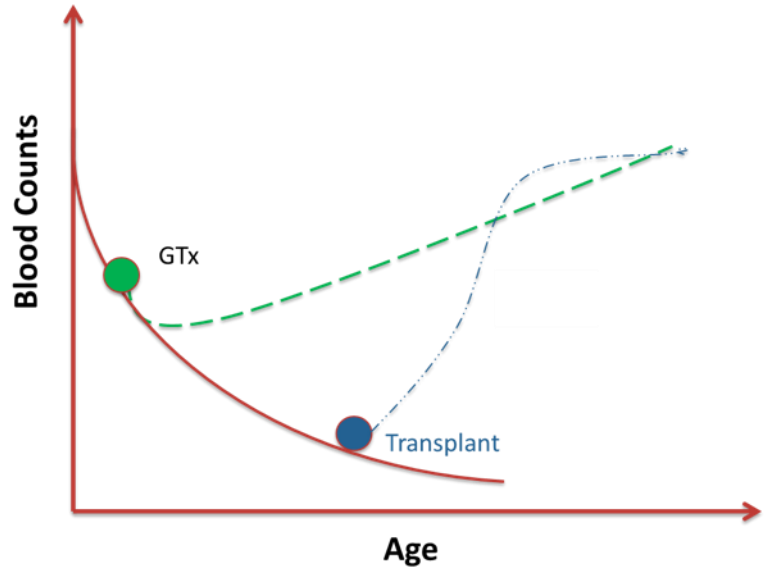
Rationale for GTx in FA:

- Somatic mosaicism demonstrates that a ***modest number*** of gene-corrected hematopoietic stem cells can repopulate a patient's blood and bone marrow with corrected (non-FA) cells.[1,2]



Gene Therapy Value Proposition:

- Potential to ***correct*** blood & bone marrow defect ***without conditioning***
- GTx implemented as preventative measure to ***avert bone marrow failure***; BMT is indicated for patients in whom marrow failure has occurred.



[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.

FA Path to Product Registration

CIEMAT-Sponsored FANCOLEN 1 Study Process A

- Interim data (>12-month follow-up) showed evidence of durable engraftment, continued improvement in phenotypic markers and stabilization of previously-declining blood counts
- No conditioning required

OPTIMIZATION

Rocket-Sponsored Process B
(Optimized CD34 cell enrichment, transduction enhancers, commercial-grade vector and modified cell processing)

- Clinical trial of ~12 patients with sites at Stanford (US), Niño Jesús Hospital (Spain), and other leading centers in the US/Europe
- No conditioning required

BLA/ MAA

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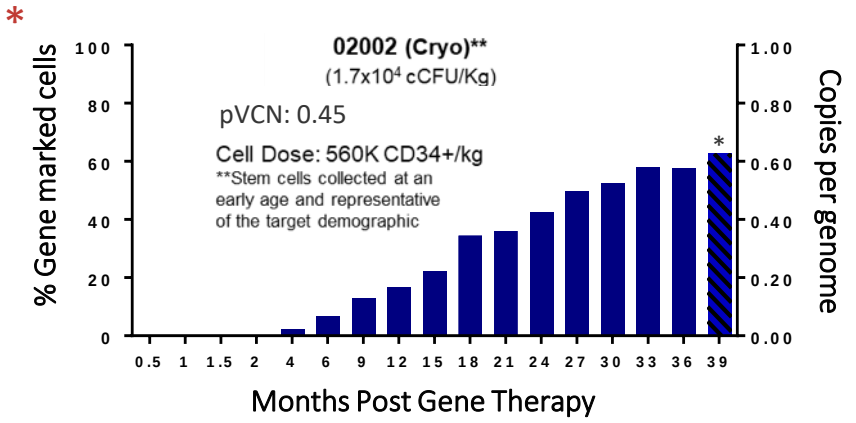
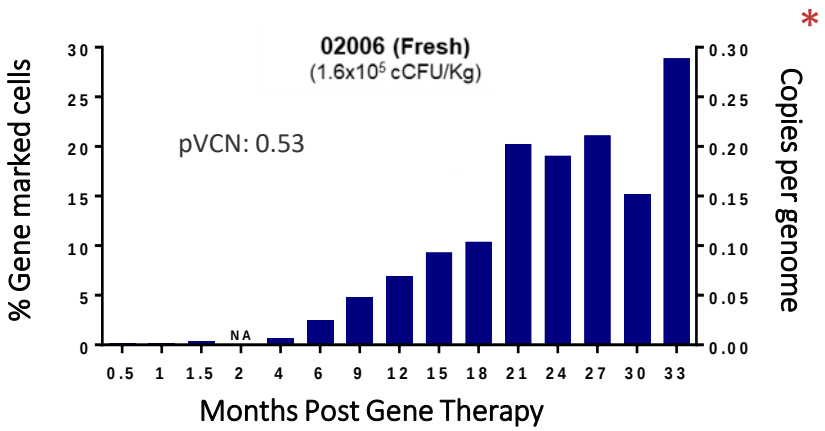
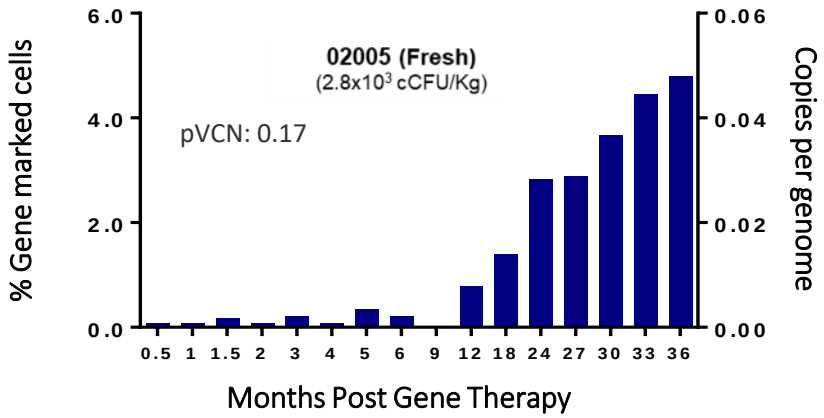
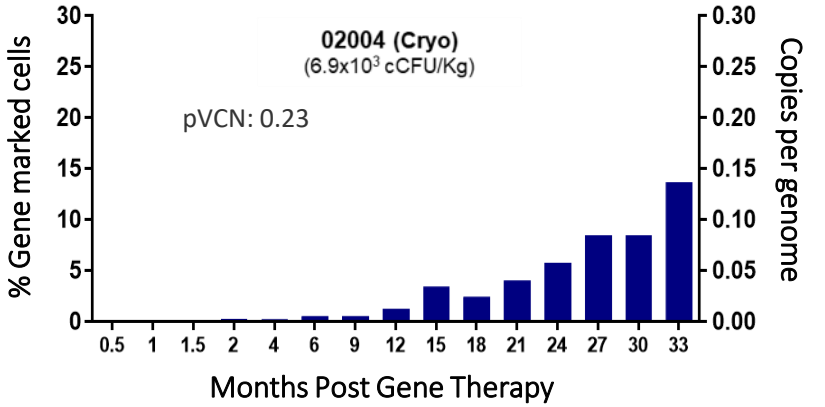
Bone Marrow Engraftment: Increasing Blood Cell VCNs Provide Evidence of Survival Advantage of Gene-Corrected FA Cells

First Demonstration of Engraftment Without Conditioning ("Process A"—non-optimized—RP-L102)









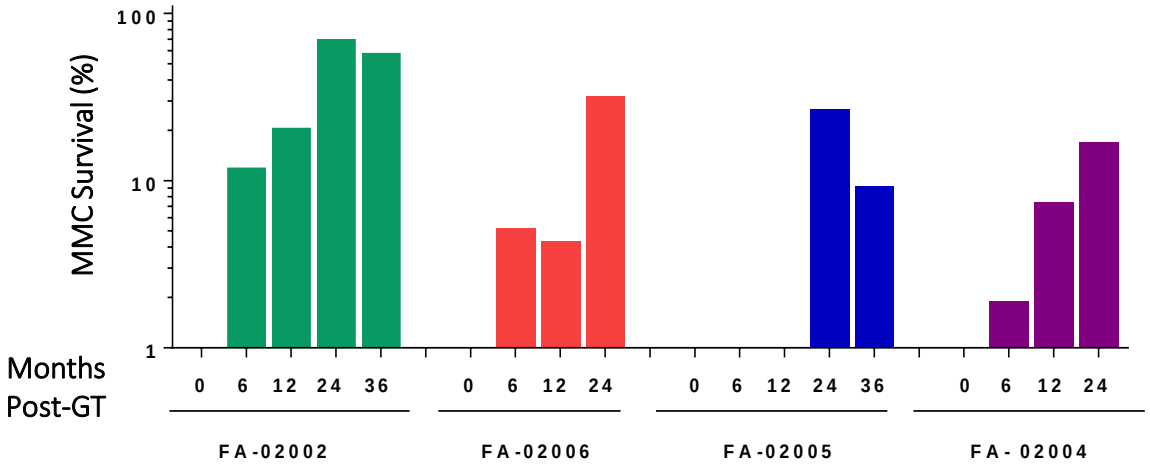
* This point requires additional validation as the long-term follow-up study is activated

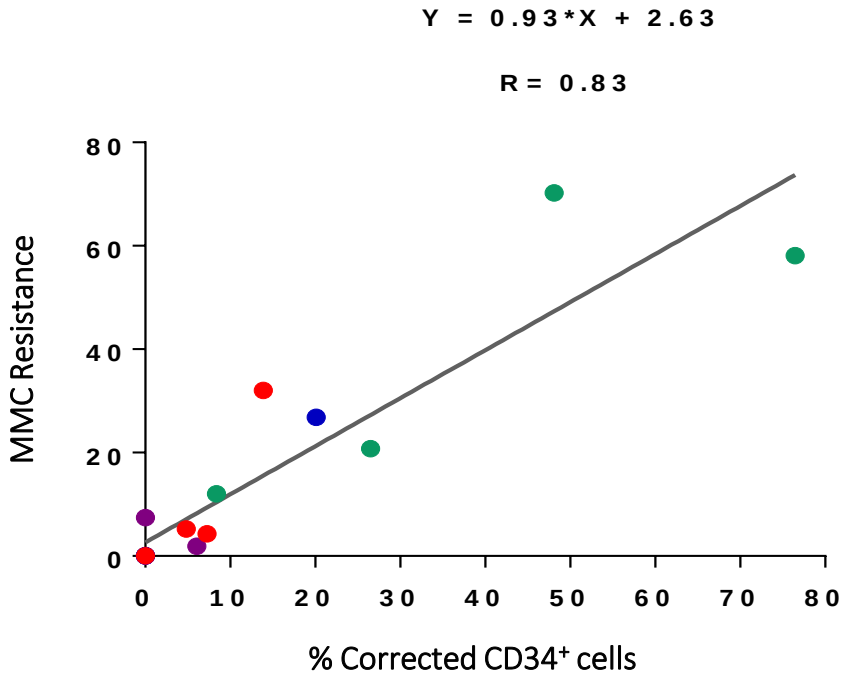
HIUNJ Data Presented at ASGCT By CIEMAT May 2020

cCFU = Corrected Colony Forming Units; pVCN: Product VCN *Minimally Acceptable Dose

Functional Correction of Bone Marrow

Progressive Phenotypic Correction of BM Cells (MMC-Resistance)



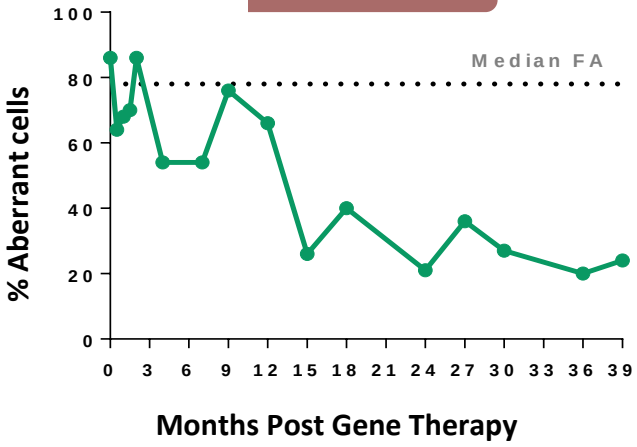


- MMC assay ***identifies cells resistant*** to Mitomycin-C (MMC), a DNA damaging agent toxic to (uncorrected) FA blood and bone marrow cells

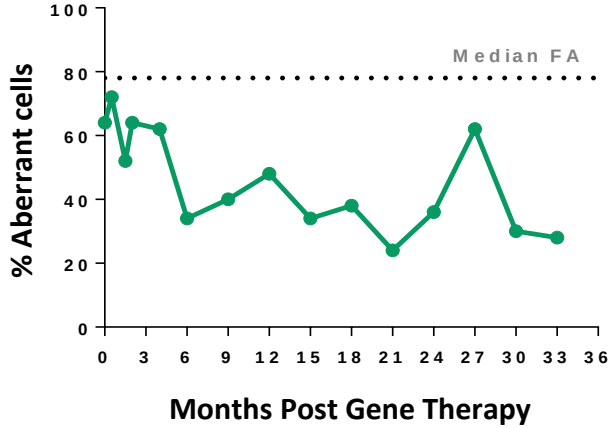
HIUNJ Data Presented at ASGCT By CIEMAT May 2020

Gene Therapy Confers a Phenotype Similar to Mosaicism

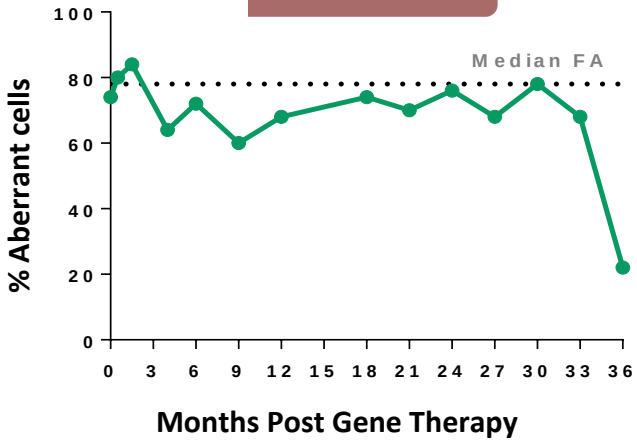
FA-02002



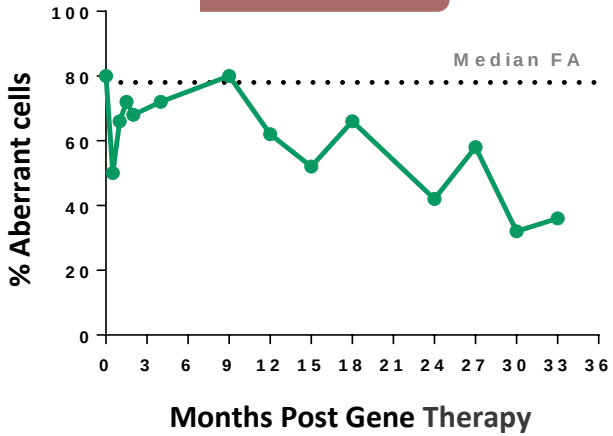
FA-02006



FA-02005



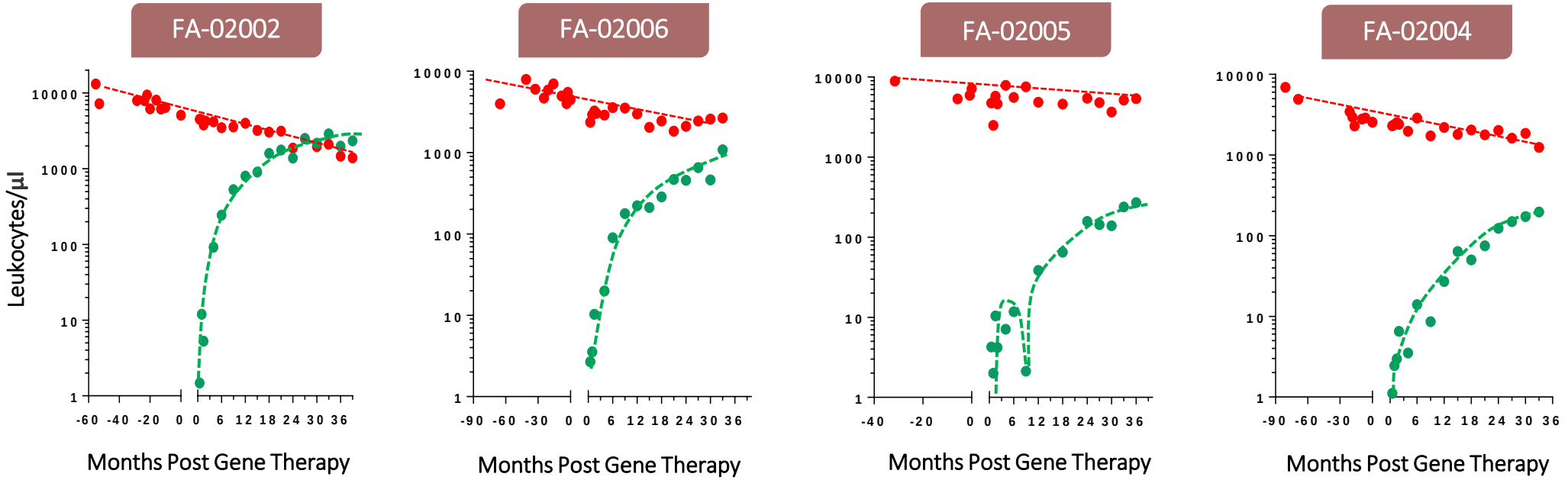
FA-02004



HIUNJ Data Presented at ASGCT By CIEMAT May 2020

Increases of Corrected Leukocytes Support Restoration of Normal Bone Marrow Function Consistent with Mosaic Phenotype

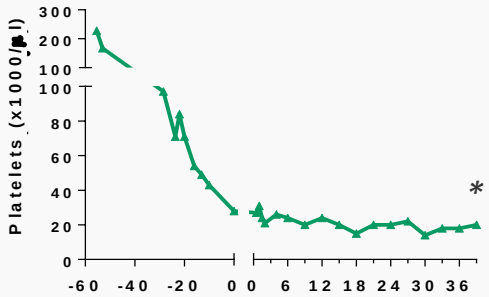
Kinetics of Corrected and Uncorrected PB Leukocytes Prior to and After Gene Therapy

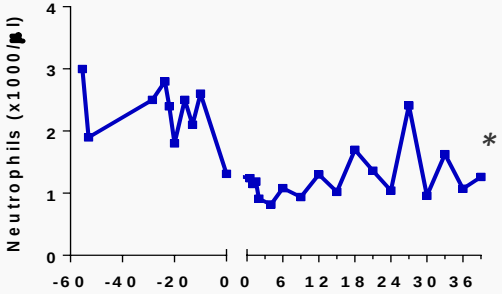


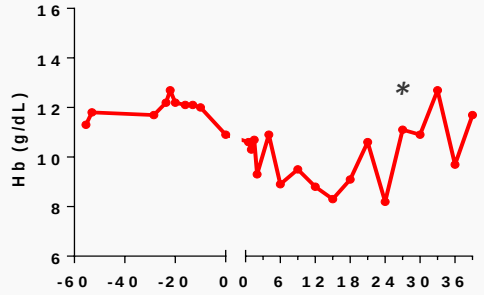
HIUNJ Data Presented at ASGCT By CIEMAT May 2020

Gene Therapy Improves Previously Declining Blood Counts in Optimally Treated Patients

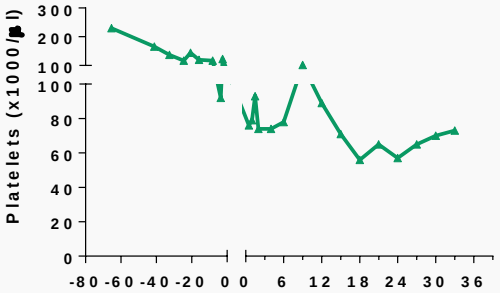
02002 (Cryo)
2.5x10^5 cCD34$^+$/Kg
1.7x10^4 cCFU/Kg

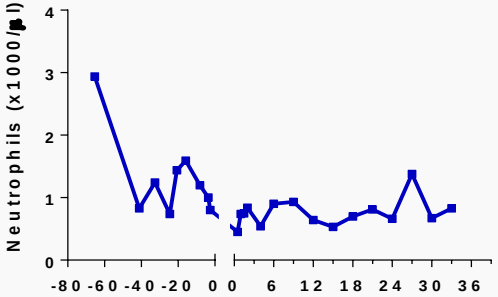


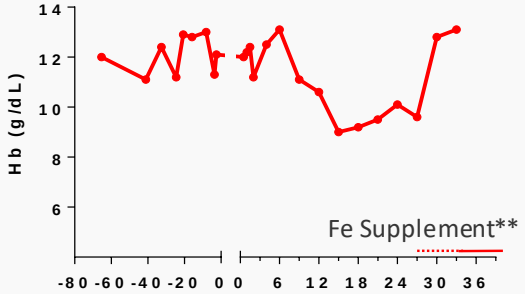




02006 (Fresh)
4.0x10^5 cCD34$^+$/Kg
1.6x10^5 cCFU/Kg







HIUNJ Data Presented at ASGCT By CIEMAT May 2020
BM = Bone Marrow; cCD34+ = Corrected CD34+ cells; cCFU = Corrected Colony Forming Units

* These particular data points require additional validation as the long-term follow-up study is activated

RP-L102 “Process B”: Pivotal Clinical Trials and Outcome Measures

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
Inclusion Criteria	Focus on patients with no/limited marrow failure, optimize preventative potential in absence of conditioning Minimum age: 1; Maximum age: US Ph 1 (12-yrs); US Ph 2 (none); EU Ph 2 (17-yrs) BM CD34+ concentration ≥ 30/µL (from aspirate); if BM CD34+ of 10-29/µL, then at least 2 of the following: Hb ≥ 11g/dL, ANC ≥ 900/µL, or Platelets ≥ 60,000/µL US Ph 1 only: At least 1 hematologic parameter (Hb, ANC or Plt) below lower limit of normal
Exclusion Criteria	Available & eligible HLA-identical sibling donor MDS or leukemia (including associated cytogenetic abnormalities) Mosaicism with stable/improved blood counts
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 ***Efficacy in 5 of 12 Patients (observed over 1-3 years post rx) required to reject null hypothesis*** **Safety** of RP-L102

Post rx = Post-treatment

RP-L102 Treated Study Patients

Phase	Patient #	Site	Age at Enrollment	Gender	Follow-up
PHASE 1	1 (1001)	US	5	F	18M
	2 (1002)	US	6	F	18M
	3 (2004)	Spain	3	M	12M
PHASE 2	4	Spain	2	F	2M
	5	Spain	3	M	2M
	6	US	3	M	2M
	7	US	5	F	2M
	8	UK	6	F	1M
	9	US	2	M	-

- 9 patients treated across 3 clinical sites, 2 under Phase 1 and 7 under global Phase 2
- All patients ≤ 6-years at enrollment
- 3 patients have ≥ 12-months of follow-up; remaining treated more recently with limited follow-up
- Note: Follow-up and patient enrollment has been complicated by COVID-19 pandemic

RP-L102 Investigational Product Metrics

Phase	Patient #	Follow-up	CD34+ Cells/kg^	CFCs/kg^	Mean VCN: Liquid Culture	Mean VCN: CFCs	Transduction Efficiency (%)	CFC Survival MMC 10nM (%)
PHASE 1	1 (1001)	18M	2.0×10^5	5.2×10^4	2.08	0.62	67	33
	2 (1002)	18M	3.7×10^5	5.0×10^4	2.21	0.92*	72	47
PHASE 2	3 (2004)	12M	4.8×10^5	1.1×10^5	1.70	0.73	100	63
	4	2M	3.2×10^6	2.8×10^5	1.65	1.56	97	62
	5	2M	1.9×10^6	1.5×10^5	2.16	0.76	61	45
	6	2M	4.1×10^6	Pending	0.62	Pending	Pending	Pending
	7	2M	2.8×10^6	Pending	1.46	Pending	Pending	Pending

Overall DP metrics were consistent with the more optimally treated patients from FANCOLEN-I study

<u>Median values</u>:
VCN (liq) 1.7
VCN (CFC) 0.76
TD efficiency 72%
CFC MMC-res 47%

Overall transduction and MMC-resistance levels in DP were consistent with high degree of corrected HSPCs

* Mean CFC VCN was assessed from a cryopreserved drug product sample.

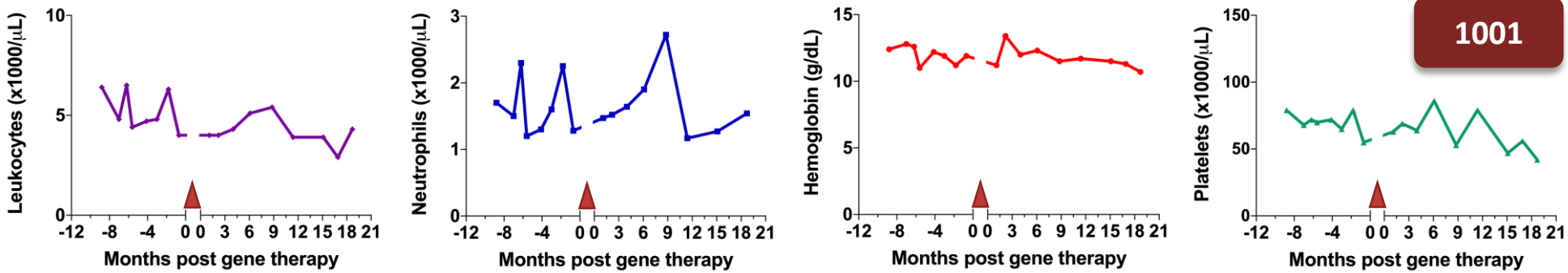
^ Per NC200 automated count (results in ~50% lower count vs. manual used in FANCOLEN-I).

CFCs: colony forming cells
VCN: vector copy number
MMC: mitomycin-C

Data as of October 2020

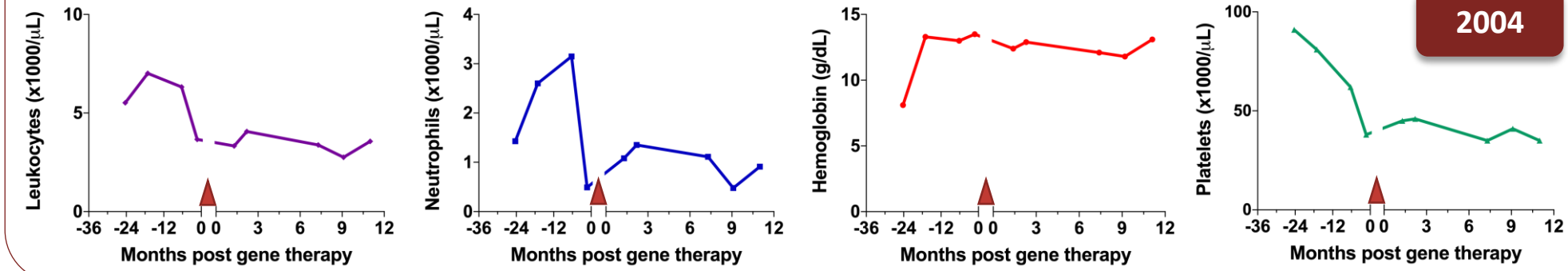
Optimal means of the nine patients treated in Fancolen-I the two that had the best benefit risk

RP-L102 Treated Study Patients (>12M Follow-up)



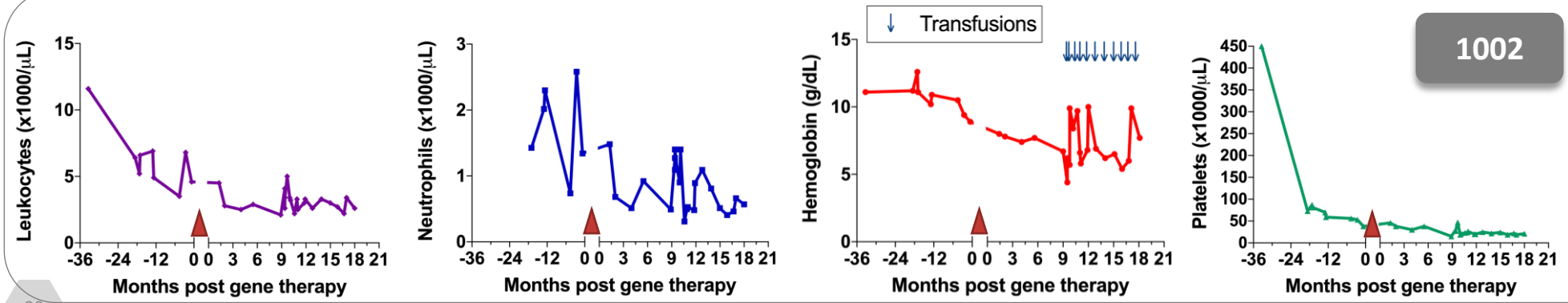
1001

- Prior to gene therapy, decreases in multiple blood lineages over prior 12-24 months



2004

- Post gene therapy, stability across multiple blood lineages observed over 12-18 months



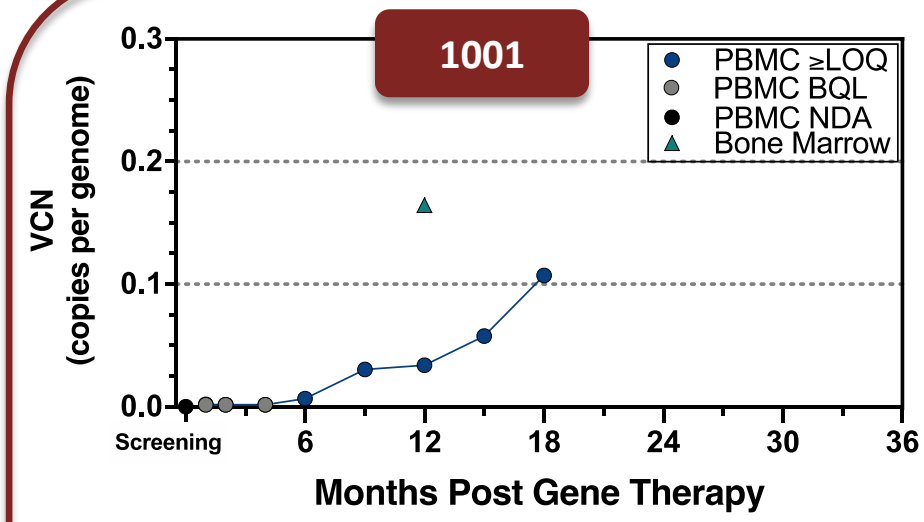
1002

- One patient with *Influenza B infection* 9-months post-rx; Required transfusions and subsequent BMT at 18-months post-rx

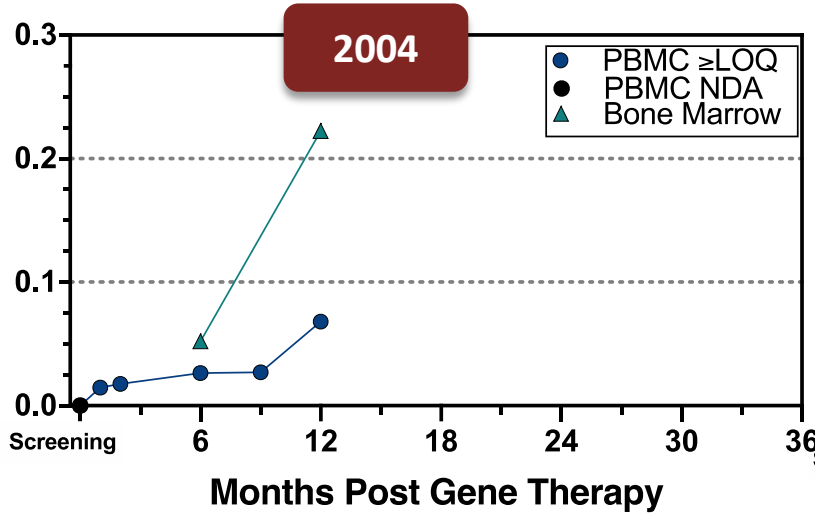
RP-L102 Treated Study Patients (>12M Follow-up)

N = 3 with ≥ 12-Months of Follow-up

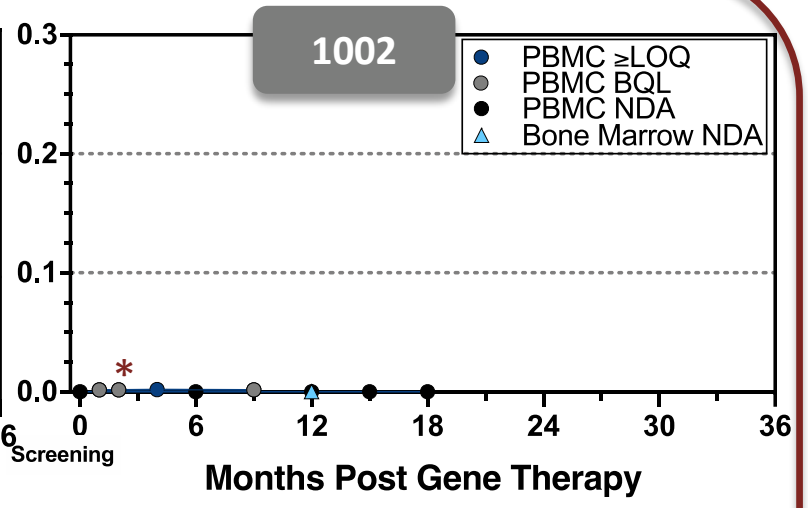
- 2 of 3 showed increasing evidence of engraftment
- 1 patient's course (1002) complicated by *Influenza B* infection; received BMT
- *Of note: Additional 3 of 4 patients with 2-months follow-up have early evidence of engraftment (0.01-0.02)*



BM progenitors resistant to 10 nM MMC at 18-months; final result pending



BM progenitors resistant to 10 nM MMC at 12-months to levels comparable to BM VCN; final result pending



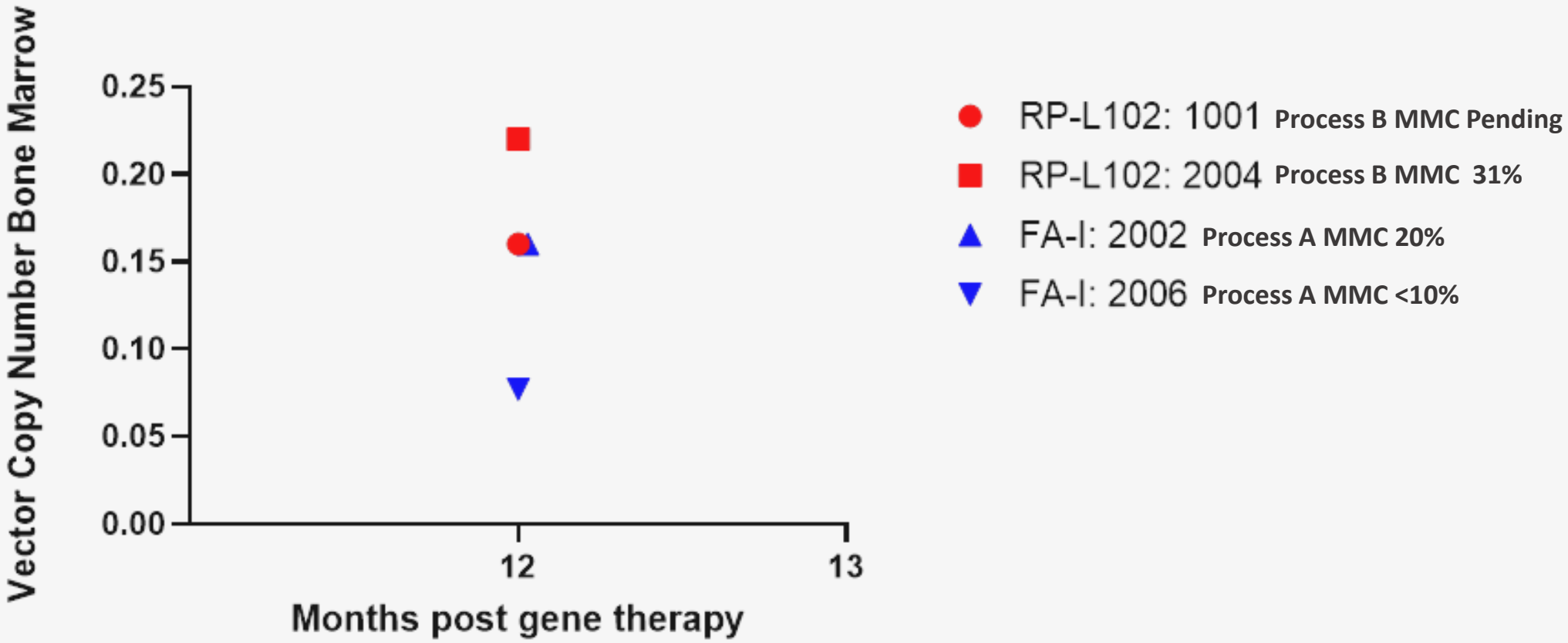
BM progenitors resistant to 10 nM MMC at 12-months suggests engraftment, but with insufficient rapidity to prevent influenza/FA-mediated BMF

LOQ = Limit of quantitation BQL= Below quantitation limits NDA = No detectable amplification

* Early time points had gene marking that was below quantification limits (BQL)

RP-L102 Treated Study Patients (>12M Follow-up)

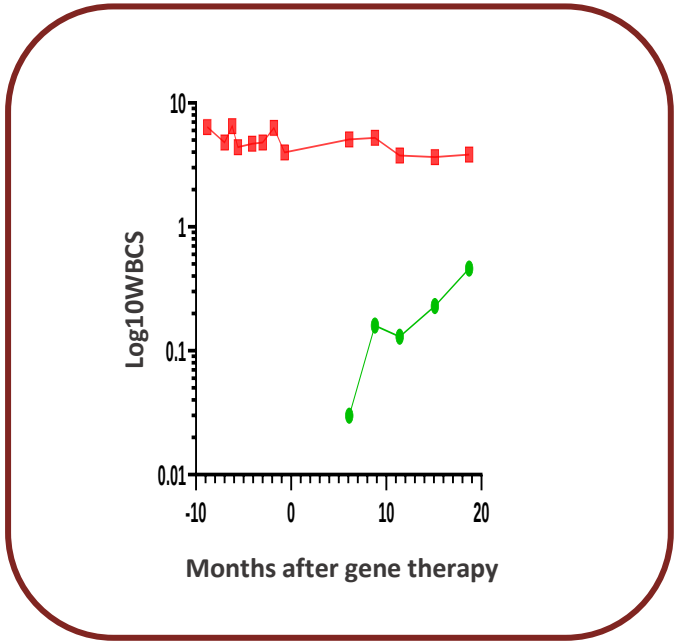
"Process B" BM VCN was in Line or Better than Optimally Treated "Process A" Patients at 12-Months



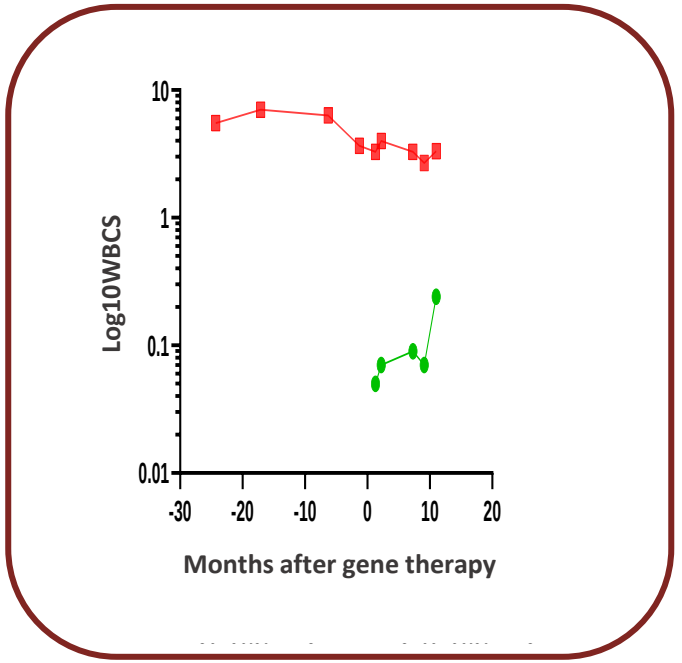
Optimal means of the nine patients treated in Fancolen-I the two that had the best benefit risk

RP-L102 Treated Study Patients (>12M Follow-up)
Increasing Proportion of Gene-Corrected Cells Observed in Peripheral Blood

Patient 1001



Patient 2004



Corrected WBC Uncorrected WBC

Summary of Pivotal RP-L102 Treated Study Patients

PB VCN available for N = 7

5 of 7 showed preliminary evidence of engraftment

N = 3 with ≥ 12M VCN

- 2 of 3 showed increasing evidence of engraftment
- 1 patient's course (1002) complicated by *Influenza B* infection; required BMT

N = 4 with early VCN (2M)

3 showed early evidence of engraftment (0.01-0.02)

- All patients clinically stable post-treatment; the patient who required BMT underwent transplant at 18-months and engrafted without complications
- RP-L102 related SAEs: 1 transient infusion-related reaction (Grade 2)
- Patient enrollment and follow-up has been challenged by COVID-19 pandemic

Data as of October 2020

RP-L102 Conclusions: Optimized "Process B" Appears to be a Consistent and Reproducible Improvement over "Process A"

- ***9 out of 12 planned patients treated*** with "Process B"
  - 7 patients with follow-up data: 3 with ≥ 12M follow-up
- Safety results appear ***highly favorable***
  - Patients treated without conditioning
  - No signs of dysplasia or other concerning features
- Evidence of ***preliminary engraftment*** observed in 5 out of 7 patients to-date
  - 1 patient's course complicated by Influenza B resulting in progressing BMF; successfully received BMT at 18-months
  - 1 patient awaiting further follow-up
- Evidence of increasing engraftment, MMC-resistance and ***stable blood counts*** in 2 out 3 patients with ≥ 12M follow-up

**** Efficacy activity in 5 of 12 patients (observed over 1-3 years post rx) required to reject null hypothesis***

Data as of October 2020

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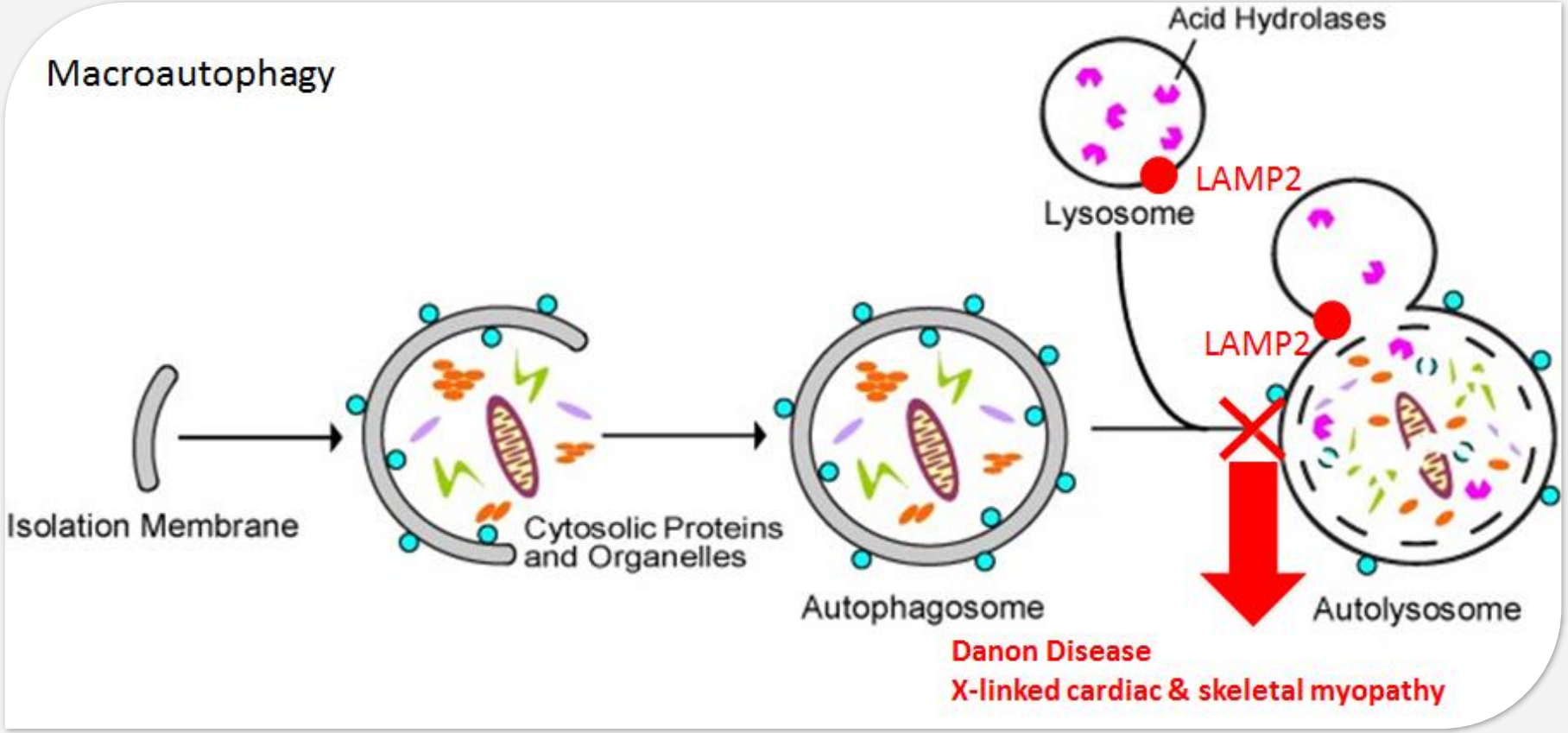
Danon Disease
Monogenic Heart Failure Syndrome

RP-L102	**RP-A501**	RP-L201	RP-L301	RP-L401
Fanconi Anemia	Danon Disease	Leukocyte Adhesion Deficiency-I	Pyruvate Kinase Deficiency	Infantile Malignant Osteopetrosis

OVERVIEW:

- **Background:** *Devastating* ***multisystemic disorder*** caused by highly penetrant and X-linked dominant ***LAMP2 mutations***, rapidly progressive cardiomyopathy is predominant cause of morbidity and early mortality in adolescents & young adults
- **Currently available treatments**: *Non-curative* heart transplants associated with considerable morbidity and mortality
- **Addressable Market:** Estimated US + Europe prevalence of ***15,000-30,000***
- **RP-A501**: AAV9 gene therapy product that elicits *improvements* in *survival*, cardiac function, and liver enzymes in preclinical studies
- **Regulatory Designations**: Orphan Drug, Rare Pediatric & Fast Track designations in the US

An Impairment in Autophagy Caused by *LAMP2B* Mutations



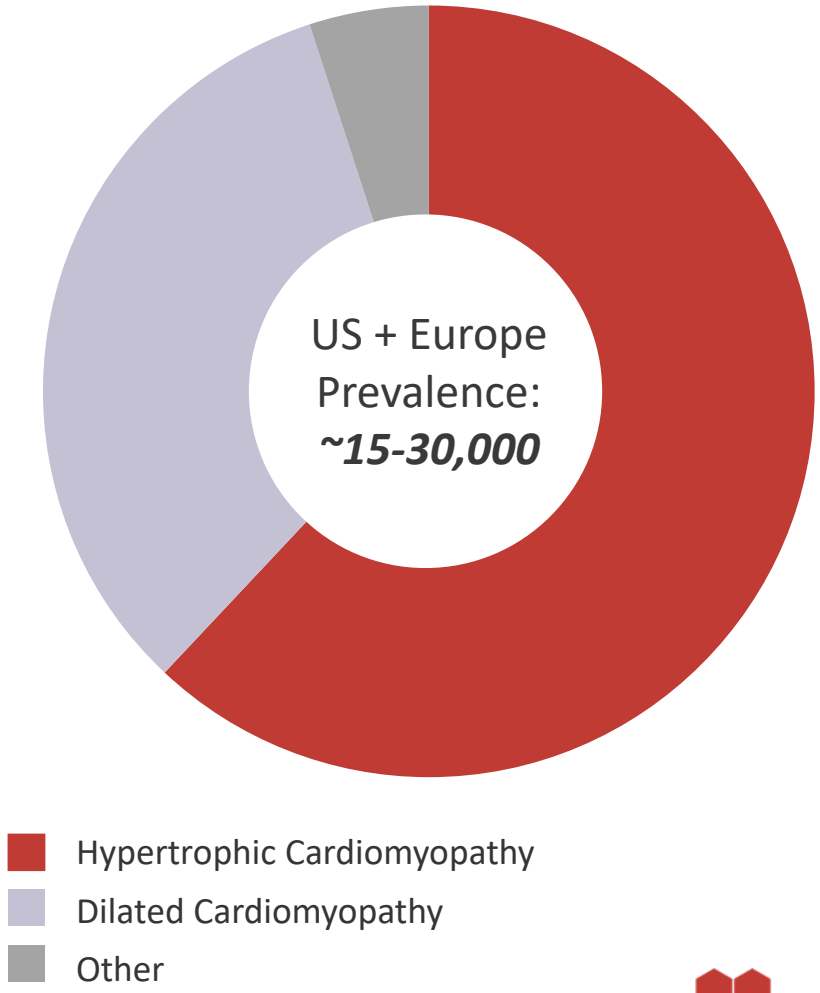
Epidemiology and Market Opportunity

Hypertrophic Cardiomyopathy (HCM)

- US HCM Prevalence: 600K-1MM+*
- 1-4% of HCM patients consistently identified with LAMP2 mutations in multiple studies with >1000 subjects evaluated**
- Danon Disease Patients with HCM: ***
  - 85% of males
  - 30% of females

Dilated Cardiomyopathy (DCM)

- Danon Disease Patients with DCM ***
  - 15% of males
  - 50% of females



* J Am Coll Cardiol. 2015 Mar 31;65(12):1249-1254.
** Heart. 2004 Aug;90(8):842-6. N Engl J Med. 2005 Jan 27;352(4):362-72. Genet Med. 2015 Nov;17(11):880-8. Gene. 2016 Feb 15;577(2):227-35. J Cardiovasc Transl Res. 2017 Feb;10(1):35-46
*** Neurology. 2002 Jun 25;58(12):1773-8. Genet Med. 2011 Jun;13(6):563-8. Rev Esp Cardiol (Engl Ed). 2018 Aug 11.

Danon Disease Causes 1-4% of Hypertrophic Cardiomyopathy:
Consistent Presence in Multiple Series Published 2004-Present

Author & Year	Age	n HCM	n Danon	% Danon	Note
Charron 2004	N.A.	197	2	1.0%	Studied LAMP2 mutations in 197 HCM patients at a general hospital in Paris
Arad 2005	12-75	75	2	2.7%	Studied glycogen storage diseases in 75 consecutive pts diagnosed with HCM (multicenter US/EU). No cases of Pompe or Fabry were detected.
Yang 2005	1m-15y	50	2	4.0%	Studied LAMP2 mutations in 50 pts with ped./juvenile onset HCM (single US center). Additional DD identified in relatives of the n=2 probands were not included in this analysis.
Cheng 2012	N.A.	50	3	2.3%	Studied LAMP2 mutations in 50 consecutive pts diagnosed with concentric LVH at a general hospital in Peking. (Concentric LVH is seen in appx. 38% of HCM). DD incidence higher (3/36) when n=14 w/ cardiac amyloidosis were removed from n=50 cohort.

Charon et al. Heart 2004; 90:842-6.
Arad et al. N Engl J Med 2005; 352;362-72.
Yang et al. Circulation 2005; 112:1612-17.
Cheng et al. Eur Heart J 2012; 33:649-56.

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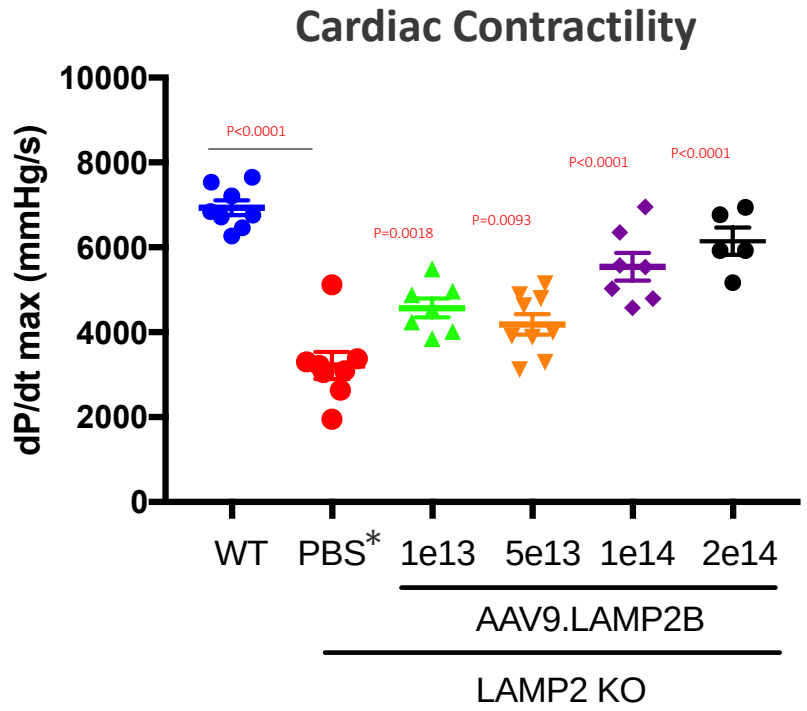
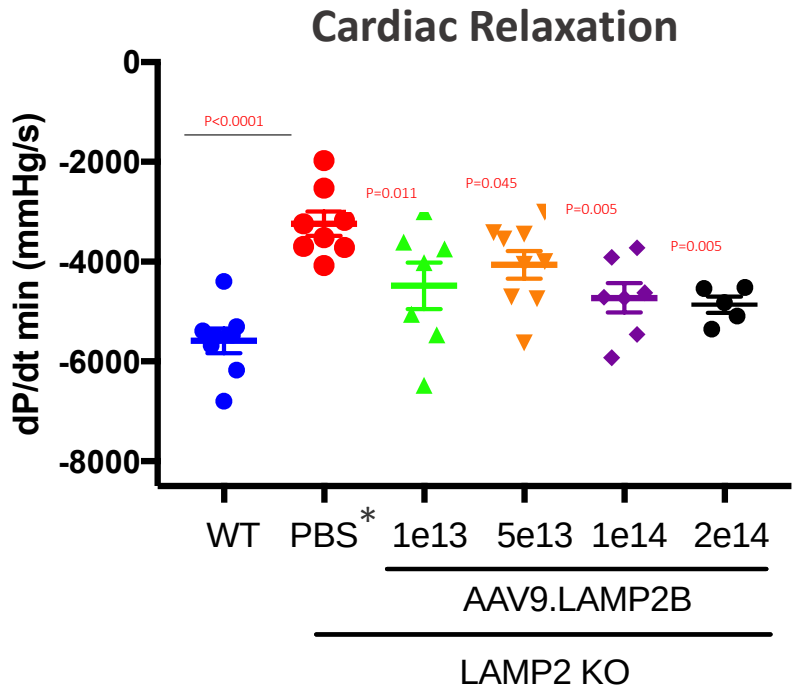
RP-A501 Restores Cardiac Function in KO Mice

Dose-Dependent Improvements in Systolic and Diastolic Function in LAMP2 KO Mice

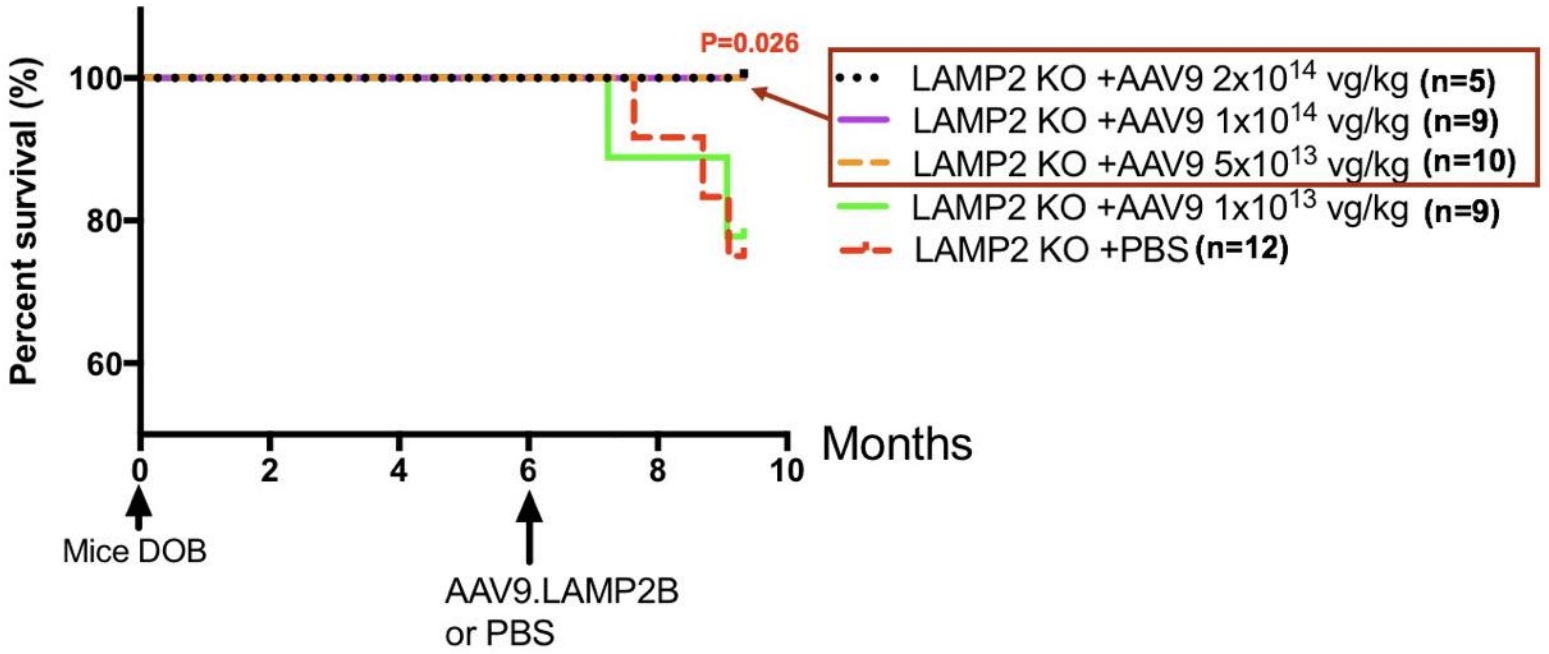




Lower dP/dt max indicates impaired contractility; Higher (less negative) dP/dt min indicates impaired heart relaxation

*PBS = Phosphate Buffered Saline (Negative Control)

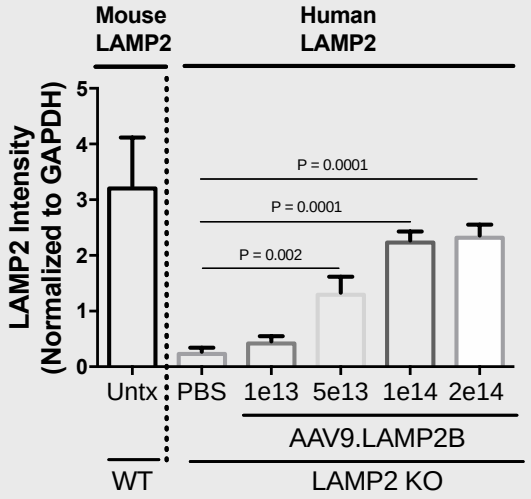
RP-A501 Shows Survival Benefit at Higher Doses in Preclinical Studies



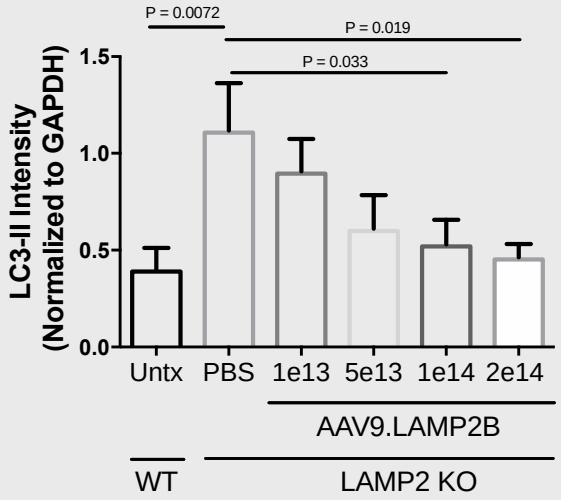
Note: All mice were sacrificed at Month 10

Protein: RP-A501 Elicits Durable Expression of LAMP2B Protein and Autophagy in Heart[1]

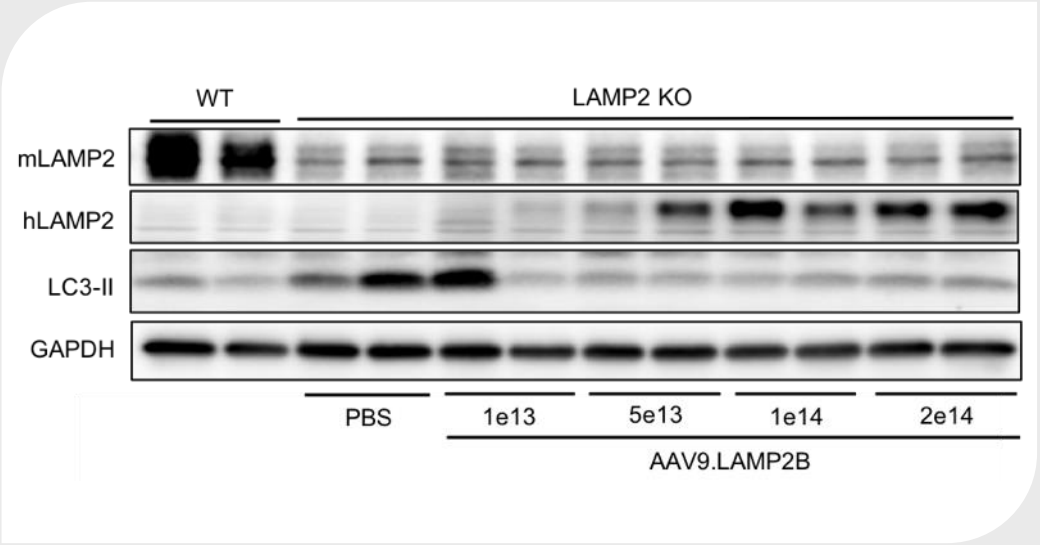
LAMP2 PROTEIN EXPRESSION



LC3-II PROTEIN EXPRESSION

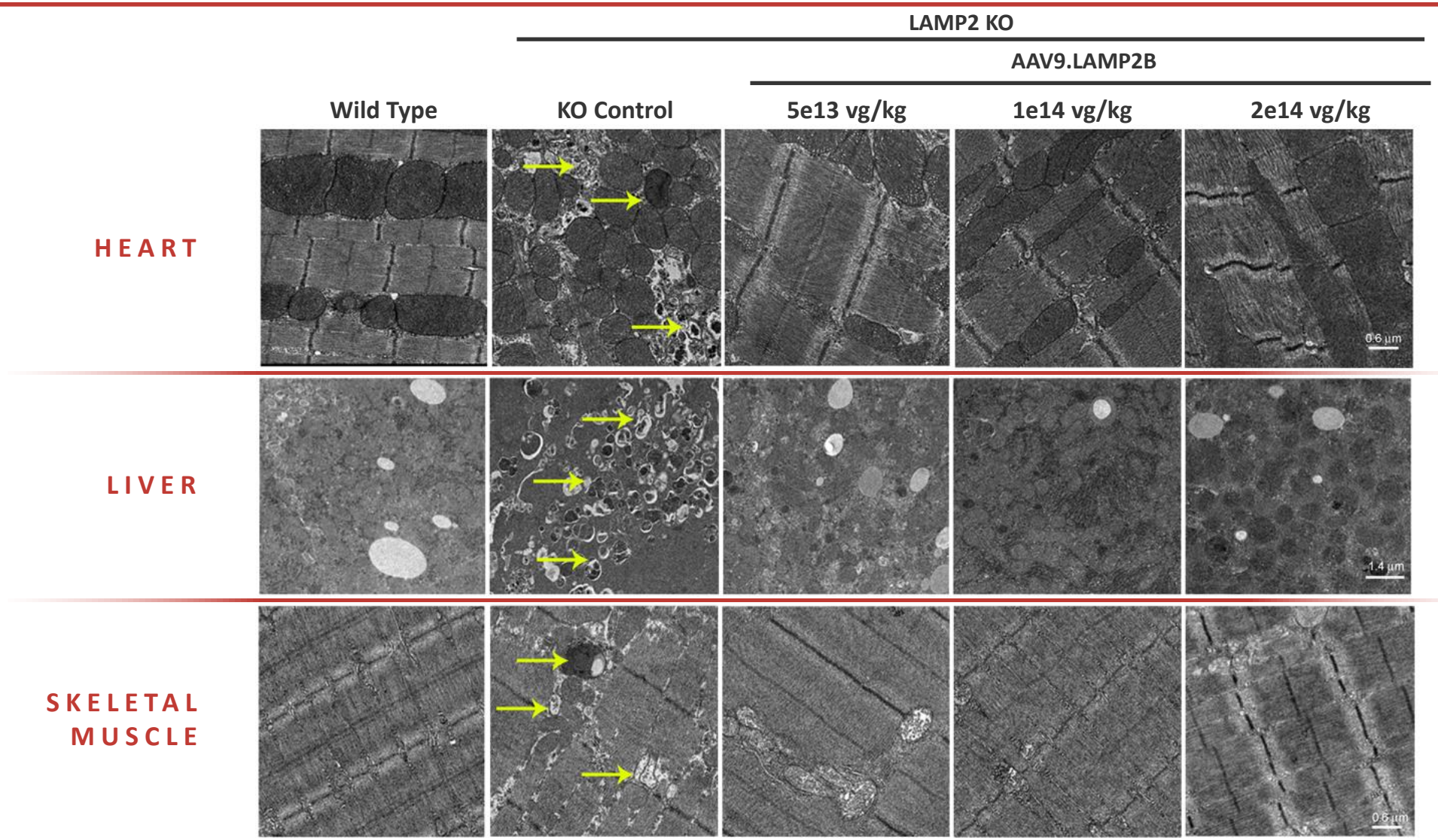


WESTERN BLOT

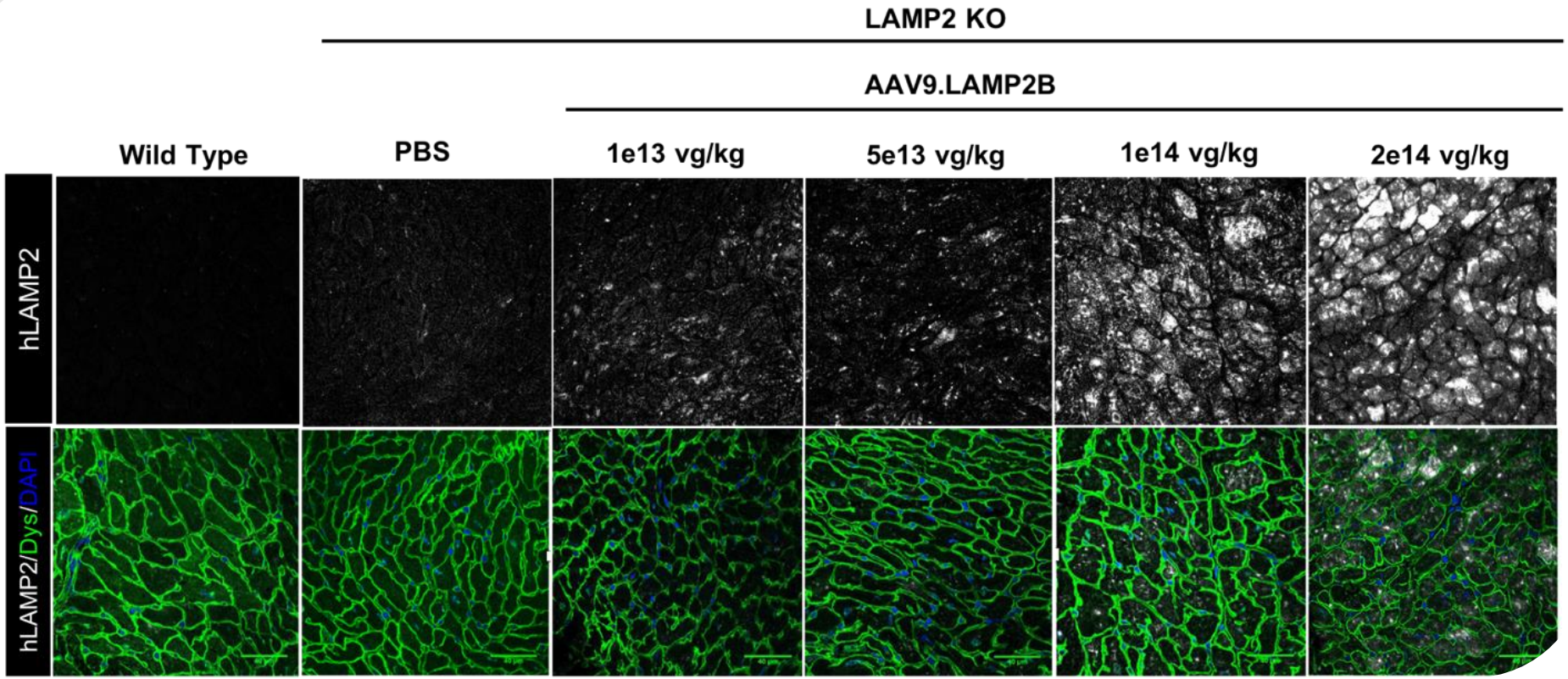


[1]Data are Mean ± SEM. N=5-8 per group. Untx = Untreated, PBS = Phosphate buffered saline
Note: Mouse LAMP2 and Human LAMP2 data are from separate Western blots.

Structural: RP-A501 Reduces Autophagic Vacuoles in All KO Mouse Models



Dose-dependent LAMP2 Expression in Cardiac Tissue



AAV9 Vector Shows Consistent Cardiac Tropism in Several Studies Across Different Species

DISORDER & VECTOR	DOSE	SPECIES	RESULTS	SPONSOR	REFERENCE
LGMD2A AAV9.hCAPN3	3E+13 vg/kg	NHP	8-80-fold higher transduction in cardiac vs. skeletal muscle	Genethon	Lostal (ASGCT 2018)
Non-specific AAV9.Luc	3E+12 vg/kg	NHP	~ 10-fold higher transduction in cardiac vs. diaphragm; and comparable to other muscle	UNC	Tarantal 2016
Pompe AAV9.hGAA	1E+11 vg/mouse	Mouse	~ 10-fold higher transduction in cardiac vs. diaphragm	U. Florida	Falk 2015
DMD AAV9.mDys	1.9 - 6.2E+14 vg/kg	Dog	2-3 fold higher transduction in cardiac vs. skeletal muscle	U. Missouri	Yue 2015
SMA AAV9.SMN	3E+14 vg/kg & 1E+13 vg/kg	Mouse & NHP	~ 100-fold higher transduction in cardiac vs. skeletal muscle (mouse)	Nationwide Children's	Meyer 2014
MPSIIIB AAV9.hNAGLU	1 - 2E+13 vg/kg	NHP	≥ 10-fold higher transduction in cardiac vs. skeletal muscle in majority of animals	Nationwide Children's	Murrey 2014
Non-specific AAV9.Luc	5E+10 vg/mouse	Mouse	5-10-fold higher transduction in cardiac vs. skeletal muscle	UNC	Pulicherla 2011
Pompe AAV9.hGAA	4E+05 - 4E+08 vg/mouse	Mouse	~ 8-12-fold higher transduction in cardiac vs. skeletal muscle or diaphragm	U. Florida	Pacak 2006
SMA AAV9.SMN	2E14 vg/kg	Human	Heart VCN ~3-4, Muscle & CNS ~1	AveXis	Kaspar 2019 (ASGCT 2019)

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Summary of Preclinical Data

- **Shows Phenotypic Improvements at Low-Dose 5e13 vg/kg:**
  - ***Survival*** benefit at higher doses
  - Dose-dependent ***restoration*** of cardiac function
  - Improvement in transaminases
- **RP-A501 *Reduces* Autophagic Vacuoles in All Examined Organs: Heart, Liver, Skeletal Muscle**
- **RP-A501 Elicits *dose-dependent increase* in LAMP2 mRNA and protein**
- **RP-A501 Preclinical Safety, Tox and Biodistribution Summary:**
  - No therapy-related deaths
  - No significant hematologic changes
  - No significant biochemical changes
  - No significant clinical chemistry changes
  - Mild and transient ALT elevation that self-resolved after one week in a single NHP
  - In both mouse and NHPs, VCN detection in Danon disease organs indicated high *LAMP2B* presence in heart tissue (for NHP, ~10x higher on average than in skeletal muscle and CNS)

RP-A501 Clinical Trial and Outcome Measures

Non-Randomized Dose-Escalation Phase 1 Study

Study Design

- Phase 1 open label study in male Danon patients
- Two age cohorts
  - Adolescent/Adult (>15 y)
  - Pediatric (8-14 y)
- Treatment doses
  - Low 6.7 x 10^{13} GC/kg
  - Higher 1.1 x 10^{14} GC/kg[1]

Primary Outcomes

- Assessment of:
  - Safety at all doses
  - Target tissue transduction & LAMP2B expression
  - Effect on cardiomyocyte histology
  - Clinical stabilization or improvement via cardiac imaging, serology and exercise testing

1. IND enables doses up to 2.0 x 10^{14} GC/kg
https://clinicaltrials.gov/ct2/show/NCT03882437?cond=danon+disease&draw=2&rank=2

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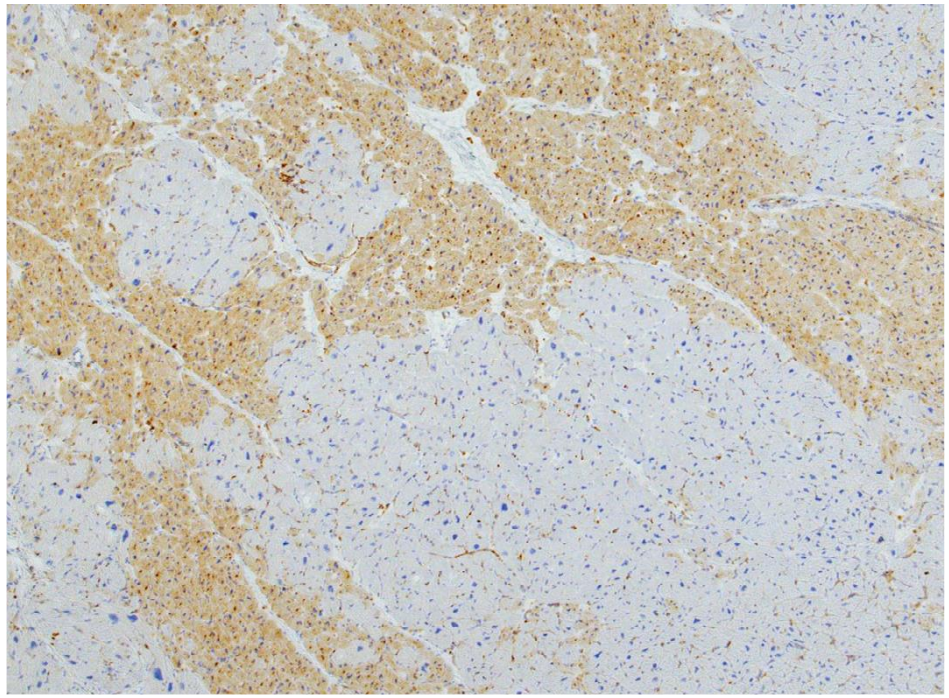
Natural History of Rapidly Progressing Heart Failure

Cardiac Clinical Features

- Progressive hypertrophic cardiomyopathy/heart failure
- Key Clinical Biomarker Changes
  - Echo:
    - Worsening diastolic parameters
      - ↑ left ventricular end diastolic diameter (LVEDD)
    - ↓ left ventricular fractional shortening (LVFS)
    - ↑ ventricular wall thickness
    - ↓ left ventricular ejection fraction (LVEF) is late event
  - Hemodynamics: Decreasing cardiac output and/or stroke volume
  - Biomarkers: Elevated BNP, CK-MB, troponin

Female Danon Cardiac Histology Suggests Broad LAMP2 Expression Important for Reversal of Phenotype

- Immunohistochemistry (IHC) from Danon female patients with severe disease display large patches negative for LAMP2 expression
- Broad expression of LAMP2 is likely the key to correcting phenotype rather than overall protein levels
- Based on this data, IHC demonstrating broad and ***homogeneous cardiac expression may be the best predictor of long-term efficacy***



Cardiac IHC Staining in Female Danon Patient Requiring Transplant at 10 y[1]

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1. *Oldfors et al 2015*

RP-A501: Subject Characteristics & AAV Vector Dose

Patient ID	Age at Treatment	Dosing Weight	Cohort Dose	Total Dose
1001	17 y	52.2 kg	6.7×10^{13} GC/kg	3.25×10^{15} GC
1002	20 y	89.1 kg	6.7×10^{13} GC/kg	5.97×10^{15} GC
1005	18 y	97.8 kg	6.7×10^{13} GC/kg	6.08×10^{15} GC
1006	21 y	82.7 kg	1.1×10^{14} GC/kg	9.10×10^{15} GC
1007	20 y	96.7 kg	1.1×10^{14} GC/kg	1.06×10^{16} GC

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RP-A501 Demonstrated a Manageable Safety Profile

- In Low-dose cohort, RP-A501 was generally well tolerated with manageable safety profile
  - ***Transient*** and ***reversible*** decline in platelets
  - ***Transient*** and ***reversible*** transaminase elevation

- In Higher-dose cohort, a single patient experienced drug-related SAE related to complement activation
  - Patient with enhanced risk due to high weight & vector dose and pre-existing AAV immunity
  - Anticipated SAE of atypical hemolytic-uremic syndrome (aHUS) resulting in reversible thrombocytopenia and acute kidney injury (AKI)
  - AKI required supportive care including eculizumab and transient hemodialysis with full return to baseline kidney function within 2-3 weeks

- ***All patients have fully recovered from immune-related sequelae***

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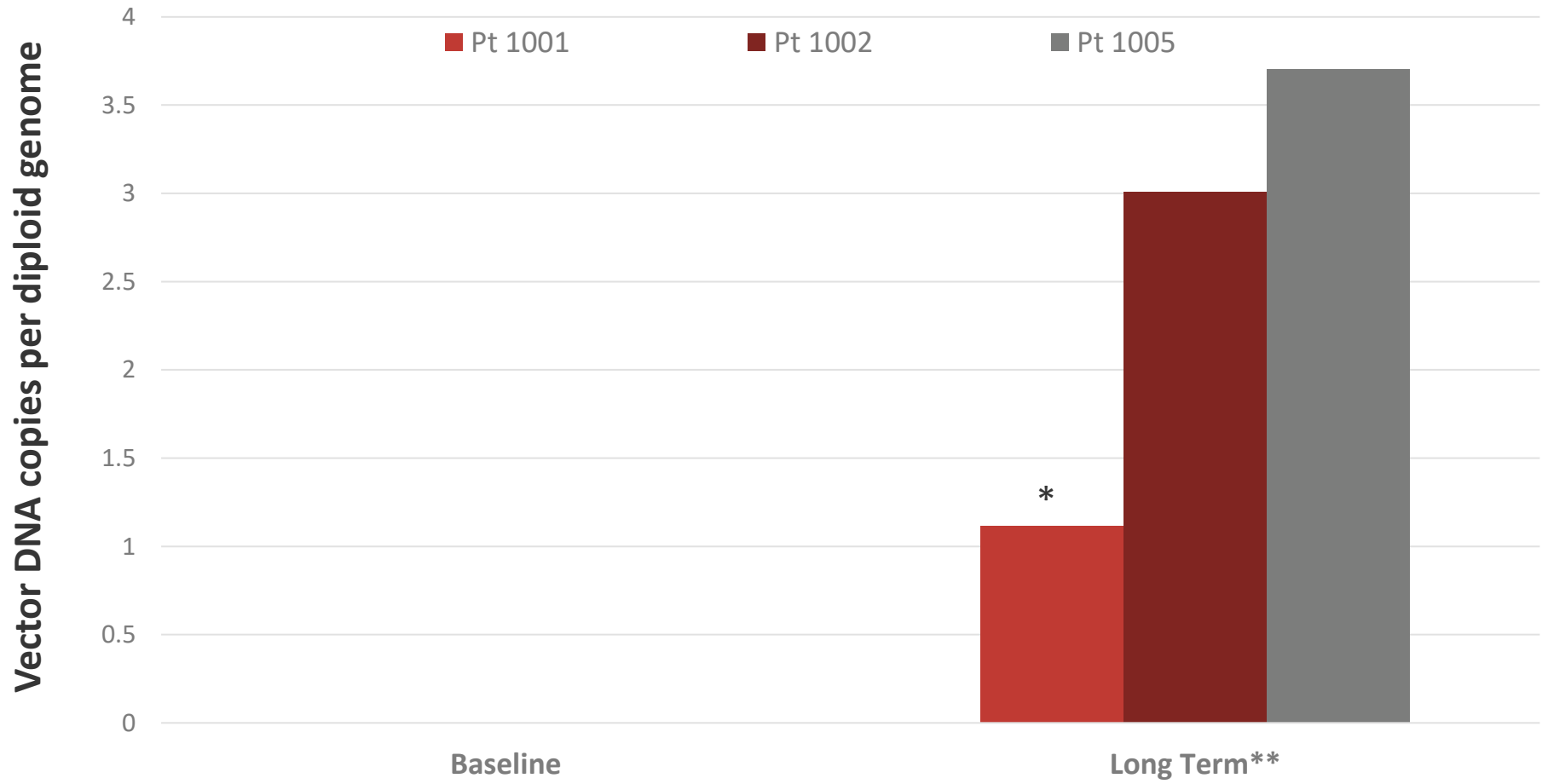
Data as of November 2020

RP-A501 Low Dose: DNA Vector Copy Number



** Clinical course and VCN drop suggest apparent poor compliance with steroid regimen*
*** 1001, 1002 Month 12; 1005 Month 9*

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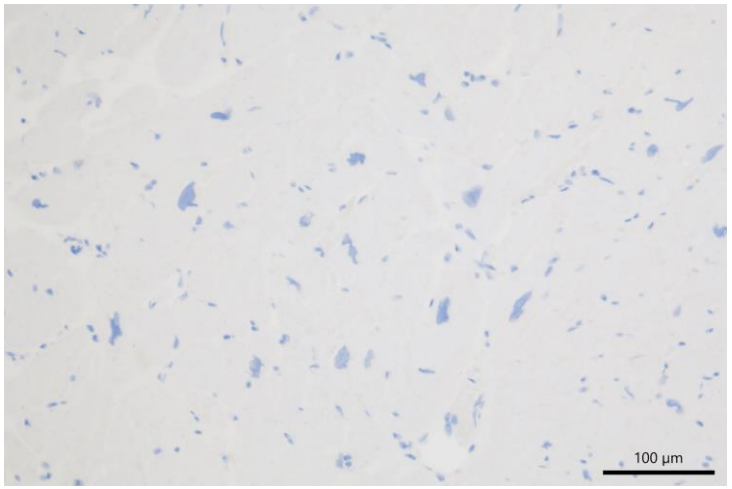
RP-A501 Low Dose Cohort Demonstrates Robust Cardiac Expression as Measured by LAMP2 Immunohistochemistry (IHC)

Patient	LAMP2B Relative Expression vs. Control*		
	Regimen	Week 8	Long Term
1001	Steroids only (limited compliance)	7.8%	<15%*[1]
1002	Steroids only (local monitoring)	36.9%	67.8%[1]
1005	Steroids → Tacrolimus	17.6%	92.4%[2]

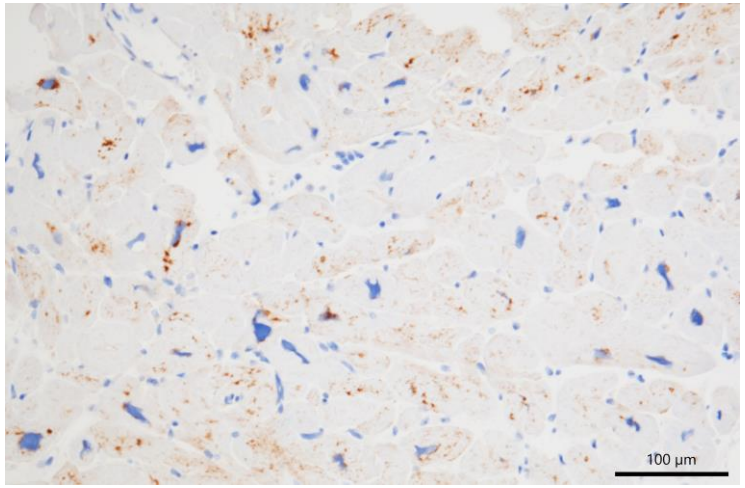
* Endomyocardial biopsies were obtained and stained for LAMP2. Percent area of cell staining was quantitated using software in a blinded fashion and expression compared to normal heart tissue. Values represent average of 3-14 sections. Qualitative assessment reported for samples with high variance.

1. Sample obtained at Month 12
2. Sample obtained at Month 9

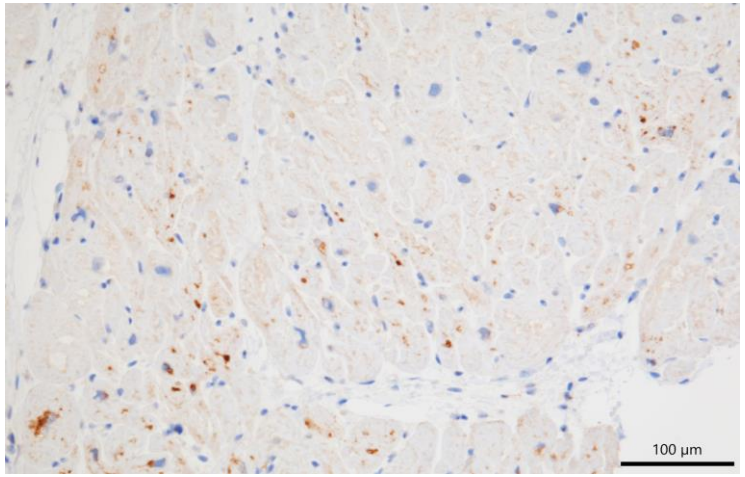
RP-A501 Low Dose: Patients 1002 and 1005 Demonstrate Robust Cardiac Expression of LAMP2 by IHC Through Months 9 and 12, Respectively



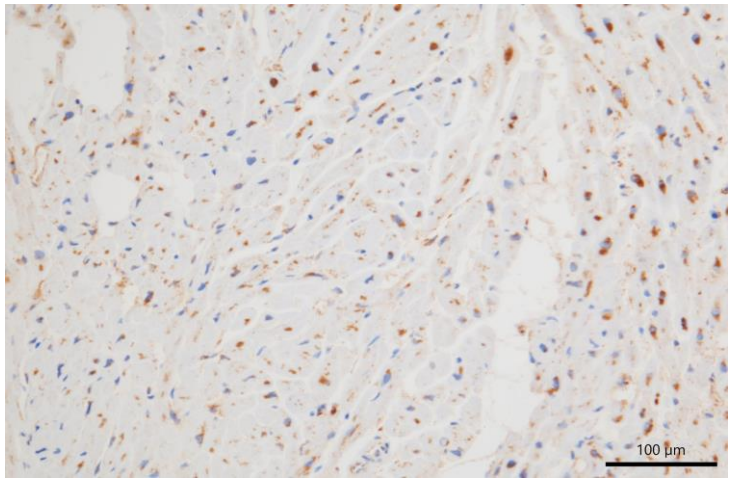
1002 Baseline



1002 Month 12



1005 Month 9



Normal Heart Control

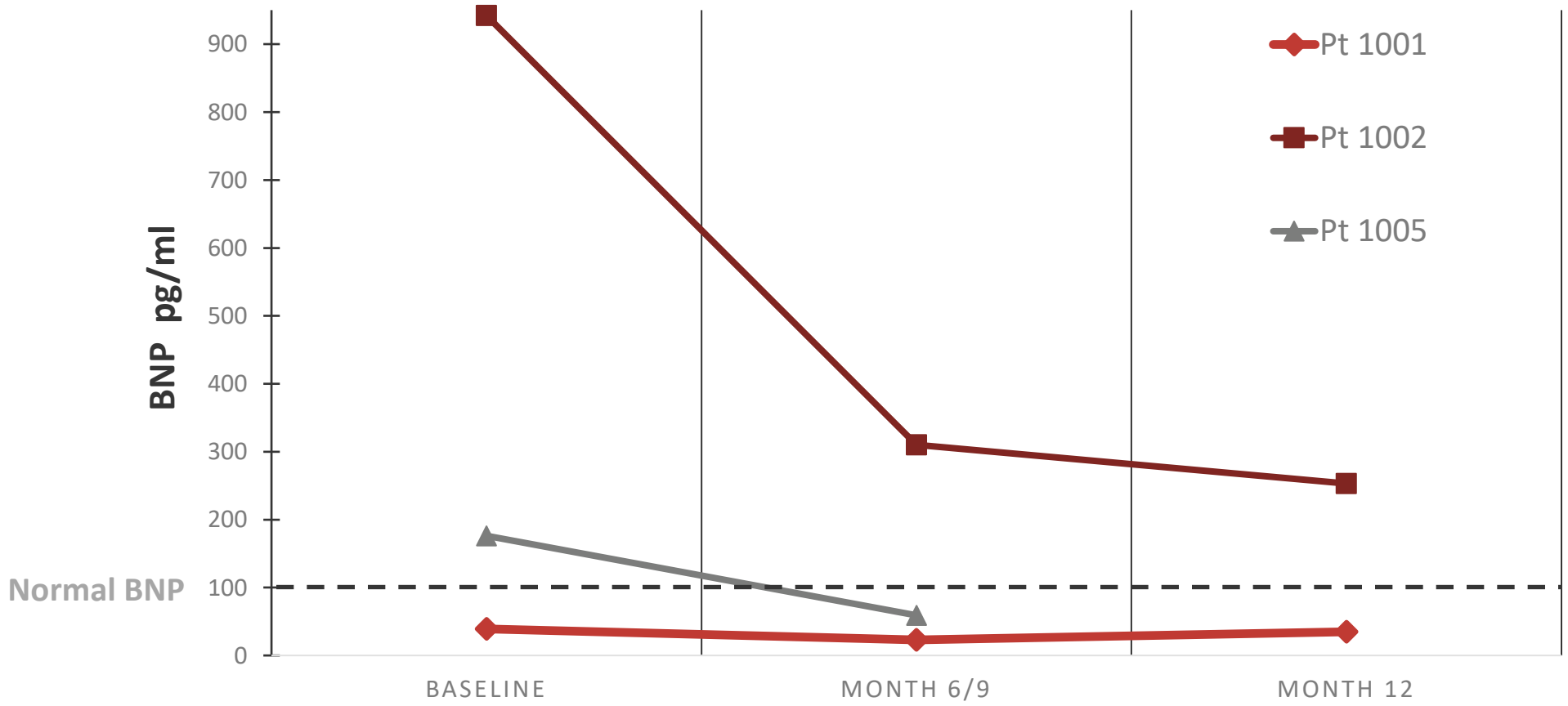
LAMP2 IHC (20x)

RP-A501 Low Dose: Endocardial LAMP2B Protein Expression

Patient	Relative LAMP2B Expression vs. Normal By Western Blot	
	Week 8	Long Term
1001	20.7%	17.9%[1]
1002	27.3%	-
1005	42.8%	61.1%[2]

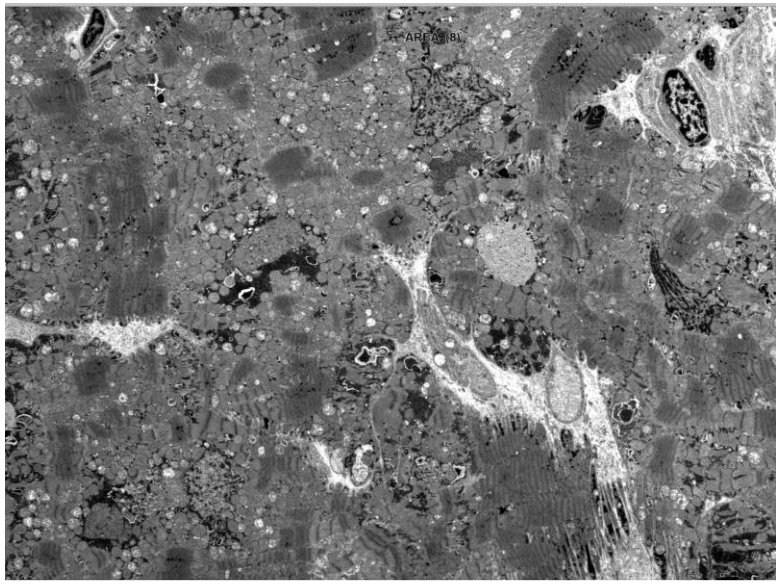
1. Sample obtained at Month 6
2. Sample obtained at Month 9

RP-A501 Low Dose Improves or Stabilizes Key Cardiac Marker of Heart Failure: B-type Natriuretic Peptide (BNP)

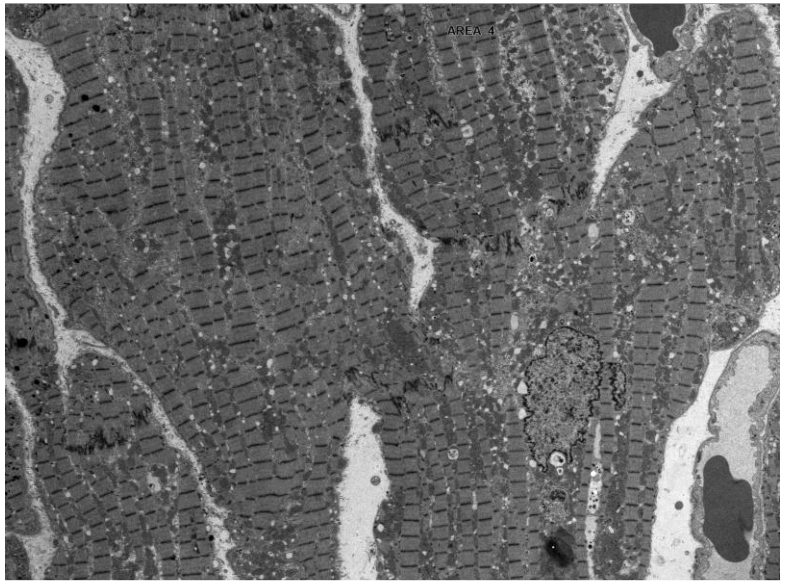


RP-A501 Electron Microscopy of Cardiac Myocytes Demonstrates Marked Decrease in Vacuolar Pathology: Patient 1005

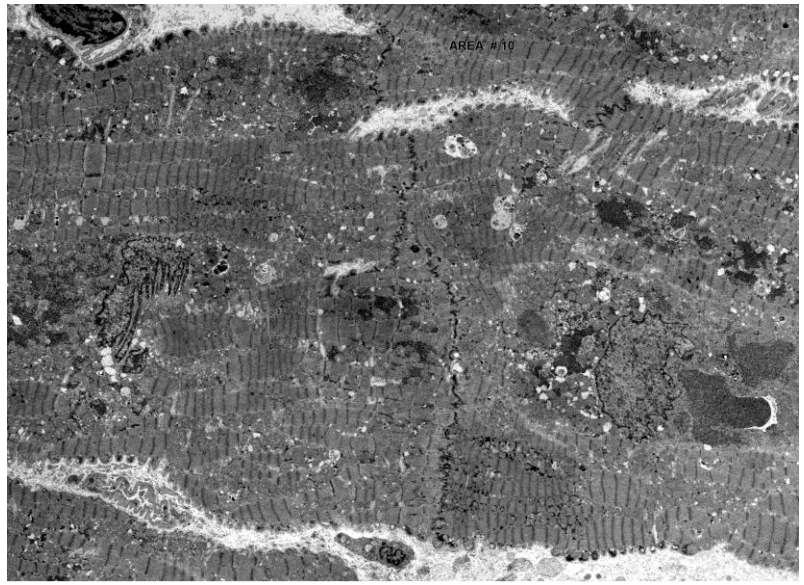
Baseline



Week 8



Month 9



RP-A501 Low Dose Confers Improvement in Cardiac Output Based on Invasive Hemodynamics in Patients 1002 and 1005

Cardiac Output (L/min)

Patient	Baseline	Long Term[1]
1001	5.2	4.12[2]
1002	3.58	5.8[2] (**1.62**x **increase**)
1005	4.5	6.08[3] (**1.35**x **increase**)

1. Calculated Stroke Volume: 40% increase in Patient 1002 and 31% increase in Patient 1005

2. Sample obtained at Month 12
3. Sample obtained at Month 9

RP-A501 Low Dose: Safety & Efficacy Findings (n=3)

- Generally, ***well tolerated*** with manageable safety profile in all low-dose patients
- ***LAMP2B gene expression*** demonstrated in cardiac biopsies from all patients
- ***Enhanced cardiac expression*** by IHC and Western blot in both patients whose compliance with transient immunosuppressive regimen was closely monitored
  - Consistent increases in percentage and level of IHC staining at later (9-12m) timepoints
- ***Positive trends*** in key biomarkers and efficacy endpoints
  - Qualitative improvement of vacuolar pathology
  - Clinical lab markers demonstrated improvement in patients 1002 and 1005
  - Trends towards stabilization and/or improvement in cardiac output
- ***Benefit observed in all three patients*** serves as clinical proof of concept as *Danon disease patients generally do not improve independently*

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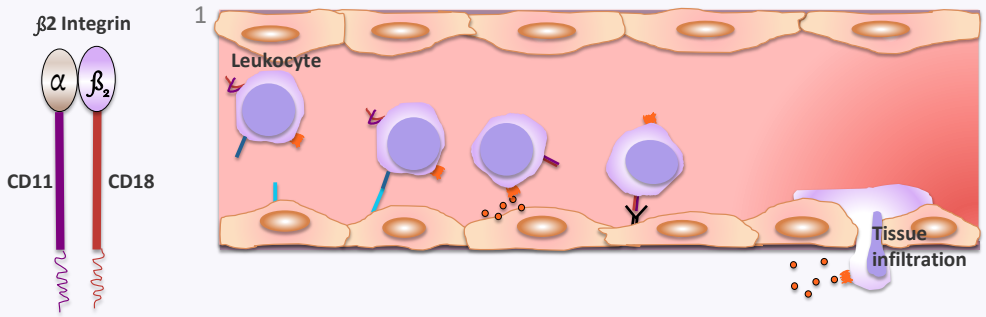
Data as of November 2020

Leukocyte Adhesion Deficiency-I (LAD-I)
Monogenic Immunodeficiency Disorder

RP-L102	RP-A501	**RP-L201**	RP-L301	RP-L401
Fanconi Anemia	Danon Disease	**Leukocyte Adhesion Deficiency-I**	Pyruvate Kinase Deficiency	Infantile Malignant Osteopetrosis

OVERVIEW:



- **Background:** Disorder characterized by 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 in the US + Europe with effective gene therapy
- **RP-L201:** Preclinical studies show stable engraftment and phenotypic correction in murine models, with restored neutrophil migration capability
- **Regulatory Designations**: Fast Track and Rare Pediatric Disease designations in the U.S.; Advanced Therapy Medicinal Product (ATMP) classification in EU; Orphan Drug designation in the U.S./EU

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

LAD-I Program Summary

Ultra-rare Disease = Streamlined Regulatory Approach

Potential design & clinical endpoints:

- **Target Patient Population:** Severe LAD-I patients (CD18<2%), ~2/3 mortality by 2y
- **Control:** Literature review of ~300 pts. (Rocket/academic collaborative publication[1])
- **Potential Clinical Endpoints**: Modest correction of CD18 expression, survival

Efficacy Trials & Registration Status – Ahead of Schedule

Registration & study planning on-schedule:

- ✓ Orphan Drug (US/EU) and Pediatric Rare Disease (US) designations granted
- ✓ IND & Phase 1/2 cleared by FDA
- ✓ Spain IMPD cleared
- ✓ US PI (UCLA Dr. Don Kohn)
- ✓ Recruitment underway from around the globe
- ❑ 3 global sites planned in the US/EU

Product/Manufacturing Optimization

Process now optimized:

- ✓ VCN using GMP vector with transduction enhancers consistently ~3 (Target VCN >1)

[1]Almarza Novoa E, Kasbekar S, Thrasher AJ, Kohn DB, Sevilla J, Nguyen T, Schwartz JD, Bueren JA. Leukocyte adhesion deficiency-I: A comprehensive review of all published cases. J Allergy Clin Immunol Pract. 2018 Jan 20. pii: S2213-2198(17)31026-7. doi: 10.1016/j.jaip.2017.12.008.

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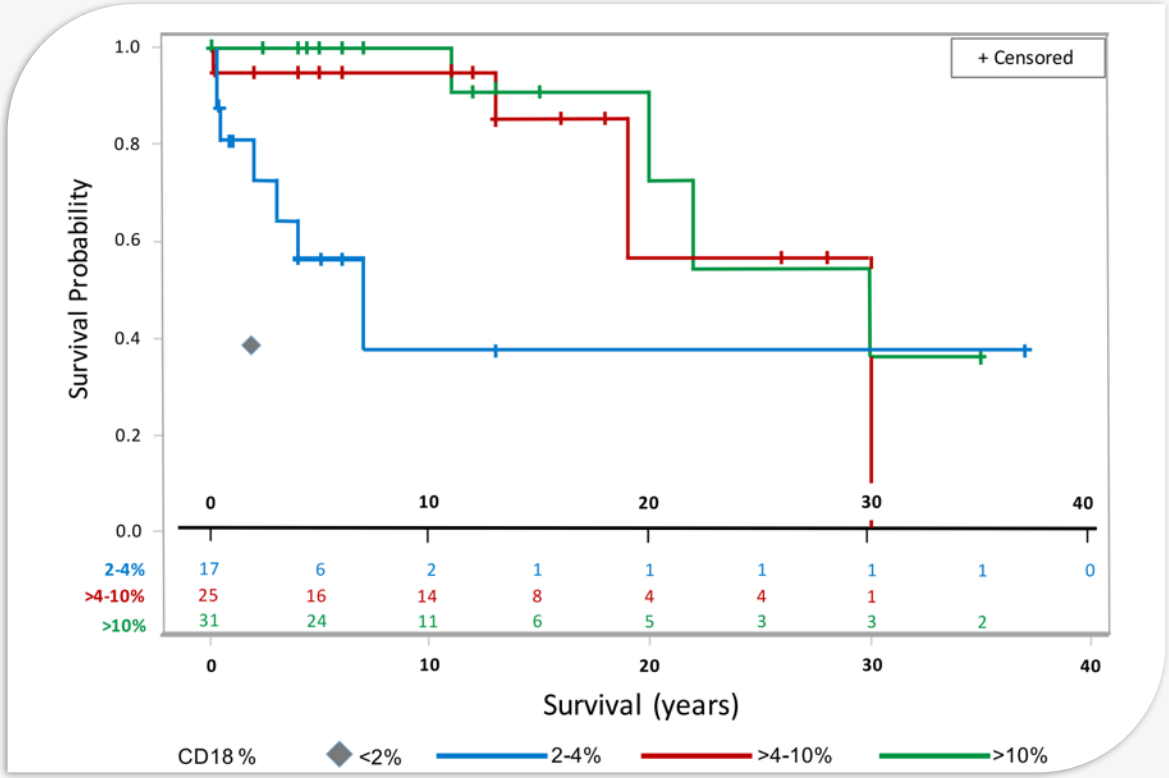
Rationale for Gene Therapy in LAD-I: CD18 Expression Correlates to Patient Survival

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

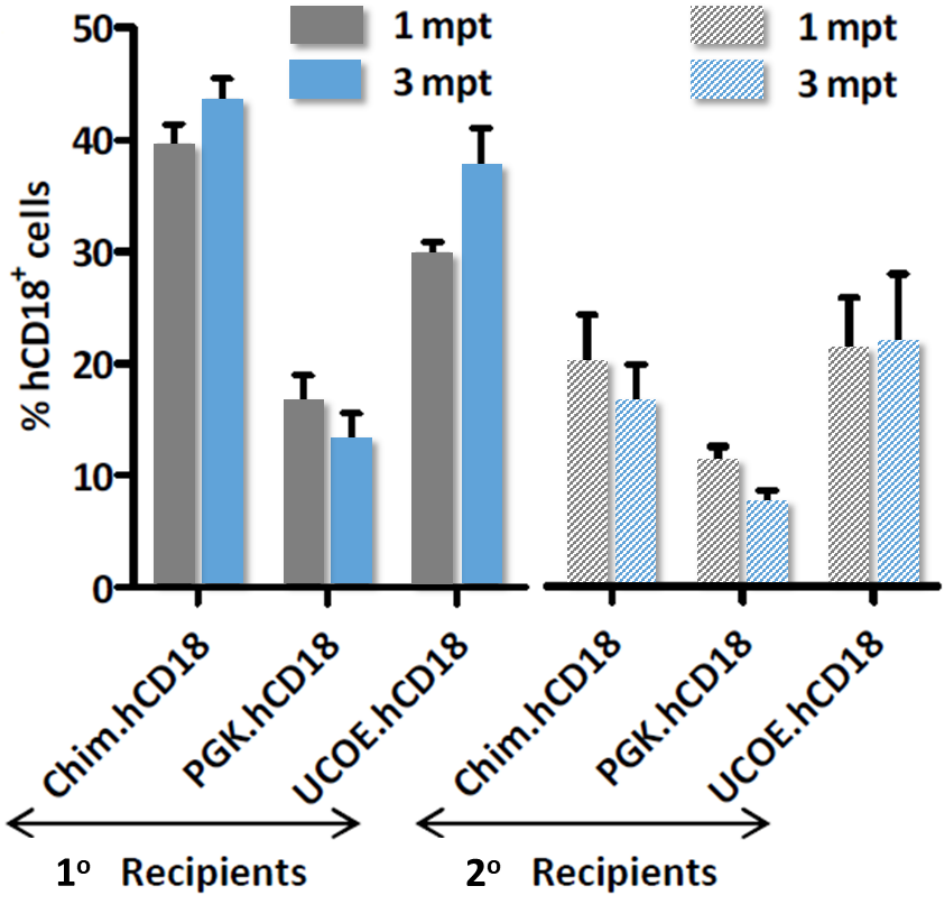
The grey diamond indicates the 39% survival to age 2 years for 66 evaluable patients with severe LAD-I not receiving HSCT

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]

LAD-I: Mouse Study Shows LAD-I Correction

- Primary and serially transplanted LAD mice underwent CD18 lenti GTx with different promoters
- Myeloablative conditioning was used
- Rocket chose the Chimeric cFES/CTSG (myeloid-specific) promoter (Post-transplant PB VCN 0.4-0.9)



Leon-Rico D, Aldea M, Sanchez-Baltasar R, Mesa-Nuñez C, Record J, Burns SO, Santilli G, Thrasher AJ, Bueren JA, Almarza E. Hum Gene Ther. 2016 Sep;27(9):668-78. doi: 10.1089/hum.2016.016. Epub 2016 May 5.

RP-L201 (LAD-I) Clinical Trial and Outcome Measures[1]

Non-Randomized Phase 1/2 Study

Design

- Enroll 9 pediatric patients globally
  - Phase 1: Enroll two patients to assess safety and tolerability
    - Enrollment Complete
  - Phase 2: Overall survival at multiple sites (US and Europe) n=7

Primary Outcomes

- Phase 1:
  - Safety associated with treatment
- Phase 2:
  - Survival: proportion of patients alive at age 2 and at least 1-year post infusion (without HSCT)
  - Safety associated with treatment

Secondary Outcomes

- Percentage of patients with at least 10% neutrophil CD18 expression
- Percentage of patients with at least 0.1 peripheral WBC gene marking (VCN) at 6 months post-infusion
- Incidence and severity of infections
- Improvement in neutrophila
- Resolution (partial or complete) of any underlying skin rash or periodontal abnormalities

[1]Source: https://clinicaltrials.gov/ct2/show/NCT03812263?cond=Leukocyte+Adhesion+Deficiency&rank=5

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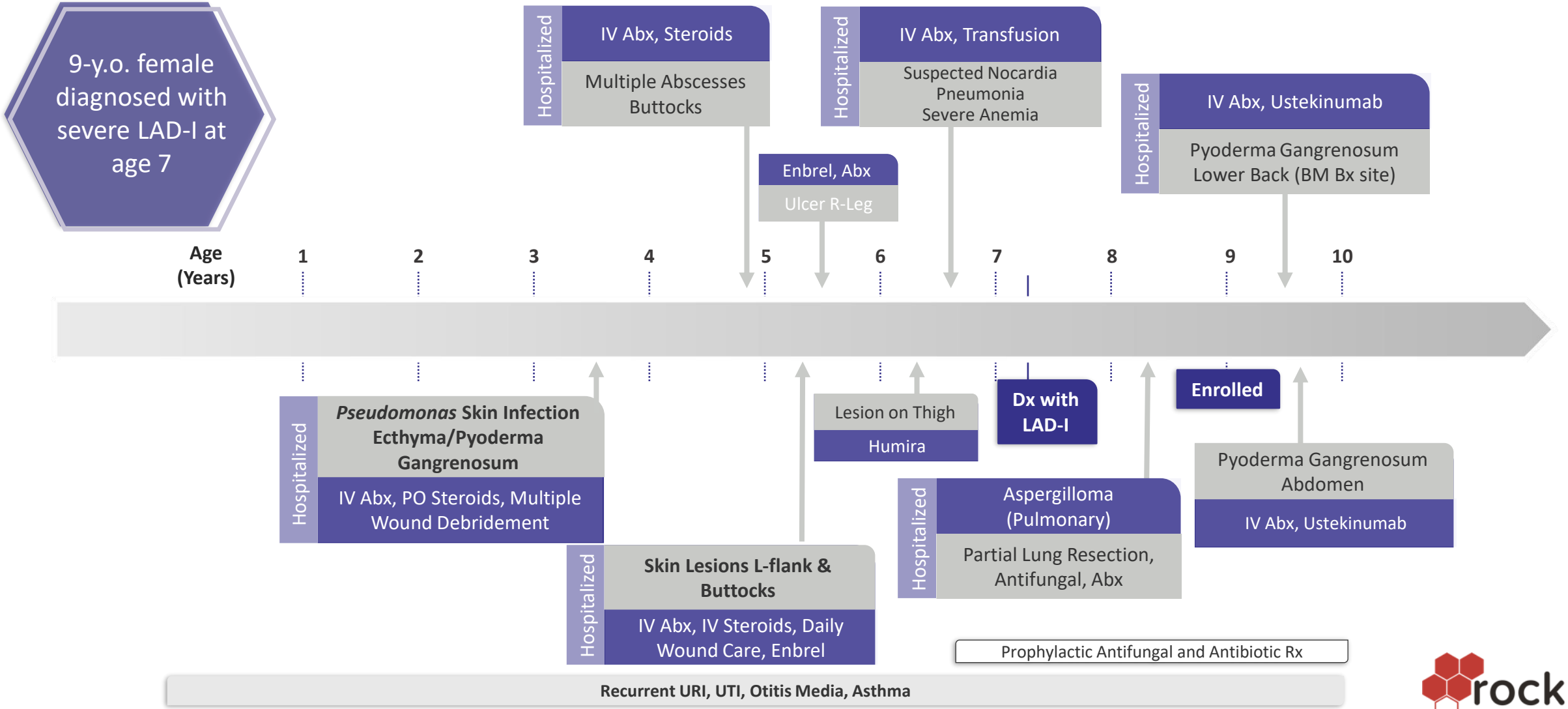
Pre-Gene Therapy Medical History of Patient 1001



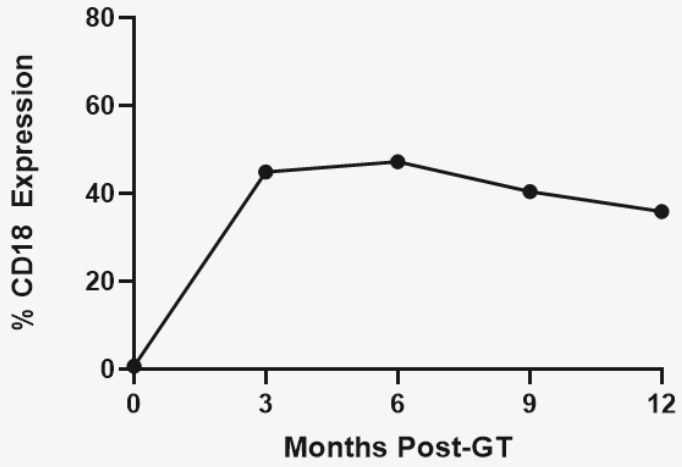
Historical patient records collected by UCLA Mattel Children's Hospital
LAD has received CIRM Funding

Patient 1001: 12-Month Follow-Up

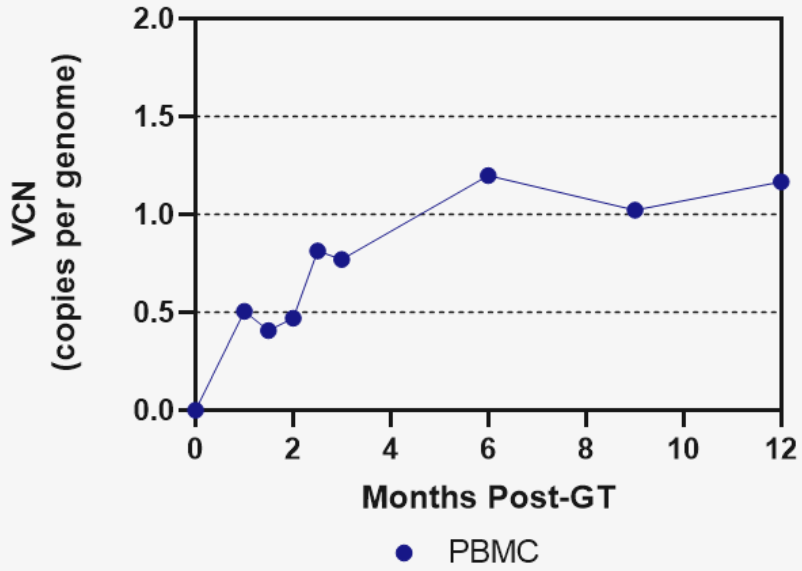
Key Drug Product Metrics

- CD34+ Cell Dose: 4.2 x 10^6 cells/kg
- Drug Product VCN: 3.8

% CD18 Expression (PMN)



VCN (PBMC)



PMN: polymorphonuclear lymphocytes

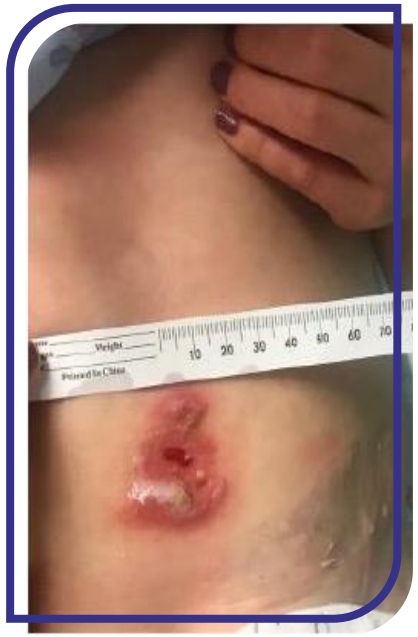
PBMC: peripheral blood mononuclear cell

Patient 1001: Visible Improvements Post-Treatment

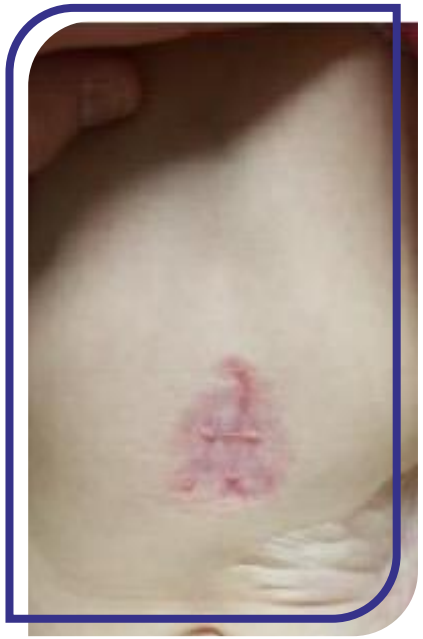
Pre GTx: Severe infections ≥ 1 per year; numerous hospitalizations, severe skin lesions, continuous prophylactic antibiotics and required home schooling

Post GTx: No infections or hospitalizations, off antibiotics and able to attend school

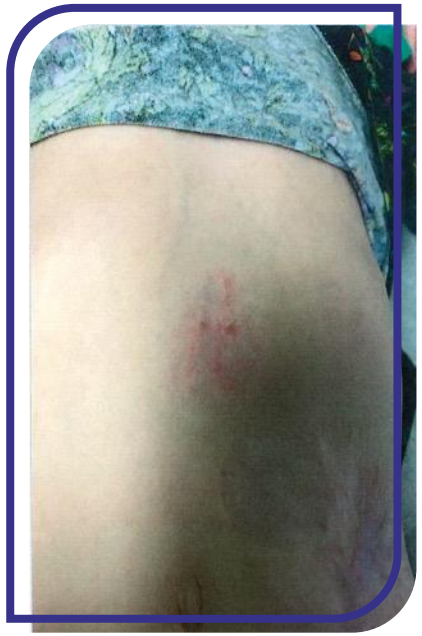
Spontaneous Abdominal Lesion



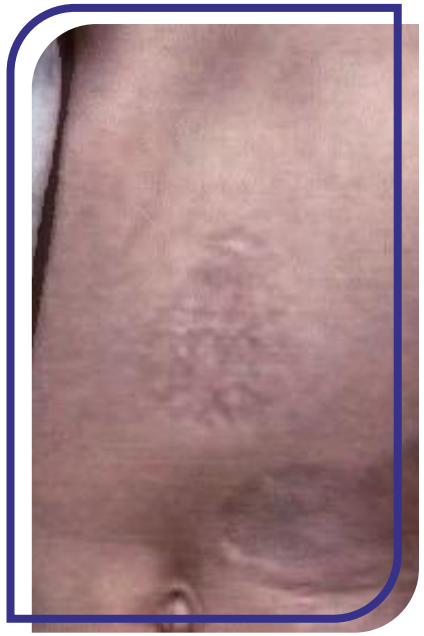
Baseline (Pre-Treatment)



3-months (Post-Treatment)



6-months (Post-Treatment)

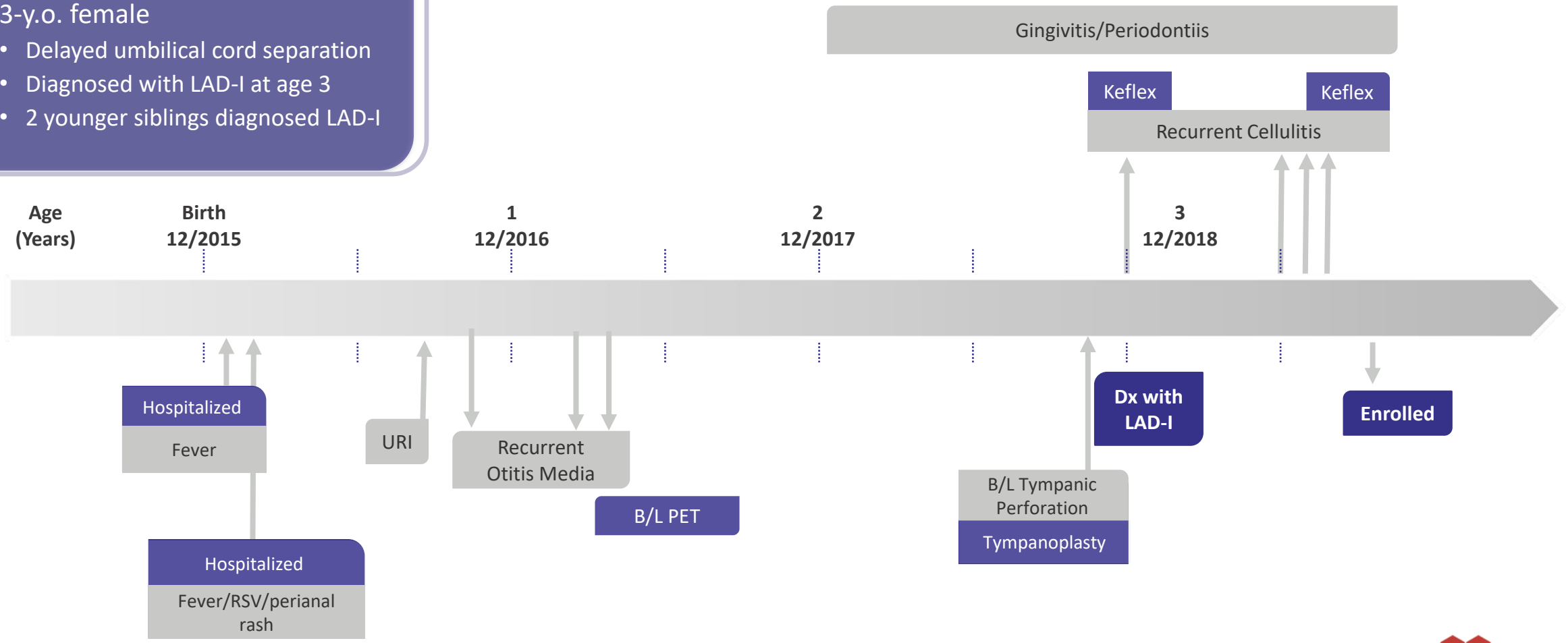


12-months (Post-Treatment)

UCLA Mattel Children's Hospital Data

Pre-Treatment Medical History of Patient 1004

3-y.o. female
- Delayed umbilical cord separation
- Diagnosed with LAD-I at age 3
- 2 younger siblings diagnosed LAD-I



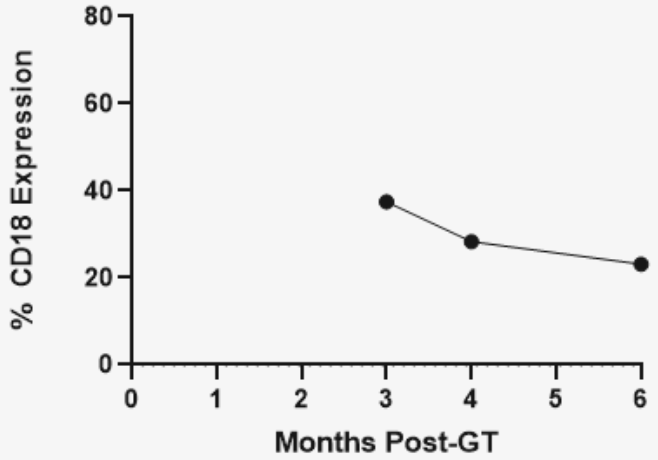
Historical patient records collected by UCLA Mattel Children's Hospital

Patient 1004: 6-Month Follow-Up

Key Drug Product Metrics

- CD34+ Cell Dose: 2.8 x 10^6 cells/kg
- Drug Product VCN: 2.5

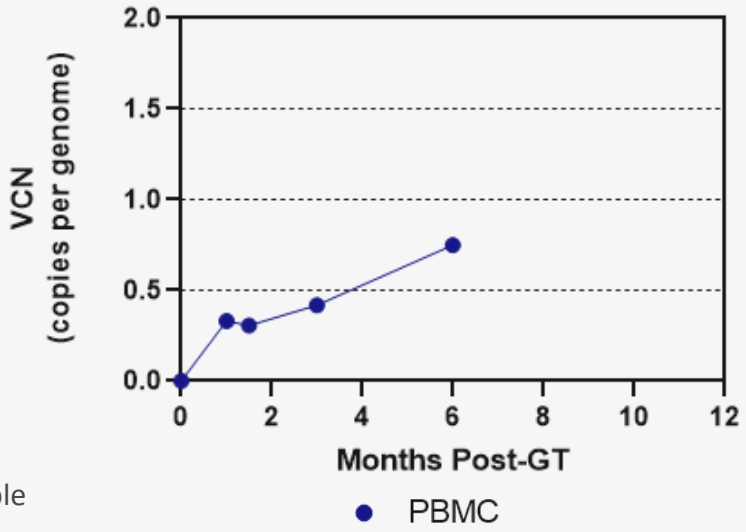
% CD18 Expression (PMN)



CD18 at baseline was reported as dim in approximately 63% PMNs, likely indicating an unstable protein, and in the context of additional clinical and laboratory evidence of severe LAD-I

PMN: polymorphonuclear lymphocytes

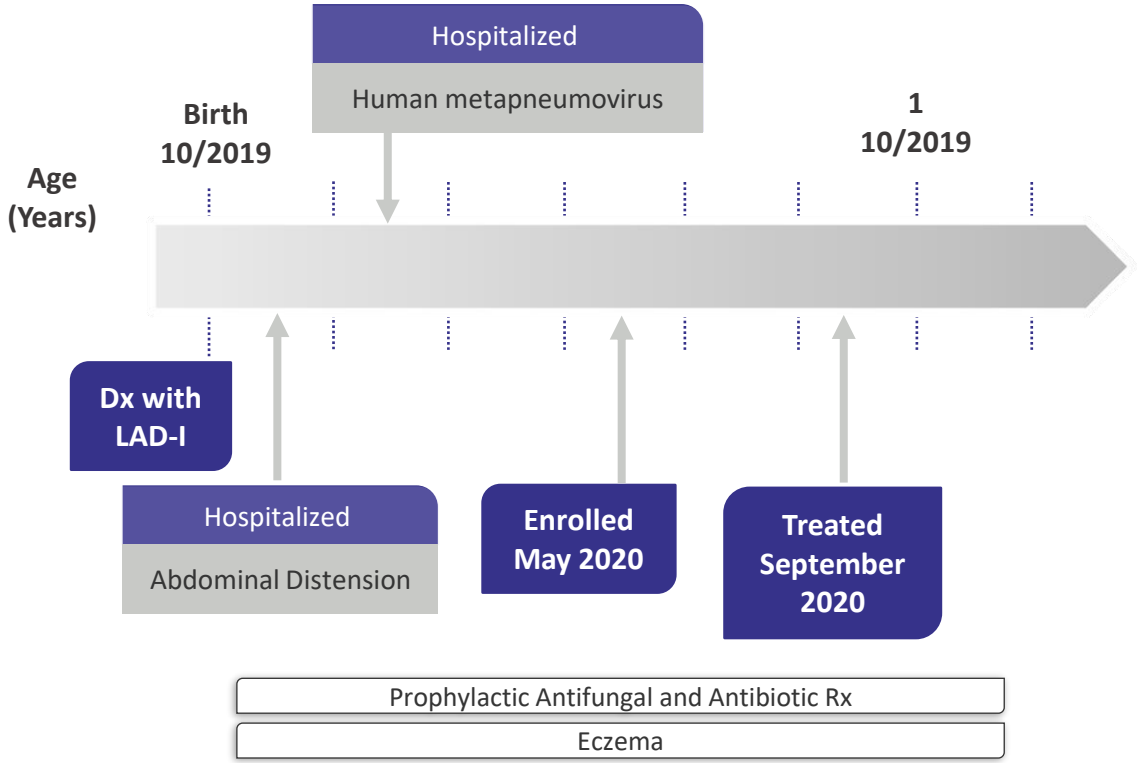
VCN (PBMC)



PBMC: peripheral blood mononuclear cell

Medical History & Follow-up of Patient 2006

7-m.o. male
- Diagnosed at birth given family history of disease
- Delayed separation of umbilical cord (6 weeks)
- 2 older siblings diagnosed with severe LAD-I



KEY DRUG PRODUCT METRICS

CD34+ Cell Dose:
4.3 x 10^6 cells/kg

Drug Product VCN:
2.87

Hematopoietic reconstitution observed Day 35 post-infusion

Clinically stable with no reported serious adverse events post-infusion

76% CD18 expression at 2-month timepoint

Historical patient records collected by UCLA Mattel Children's Hospital
LAD has received CIRM Funding

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RP-L201 Study Summary

- **Drug product produced in 7 out of 9 patients in Ph 1 & Ph 2**
- **Safety results of RP-L201 appear favorable**
  - Infusion well tolerated; no drug product-related SAEs or severe AEs as of November 2020
- **Preliminary Efficacy of CD18 PMN expression observed in both patients with ≥ 6-months of follow-up**
  - Patient 1001: durable CD18 PMN expression ~40% and PB VCN of 1.2 at 12-months post-infusion and ***resolution of skin lesions***
  - Patient 1004: CD18 PMN expression 23% 6-months post-treatment and PB VCN kinetics similar to those of first patient
  - Patient 2006: 2-months post-treatment had CD18 PMN expression of 76%
- **Commercial-grade drug product and centralized testing for all patients treated**
- **Phase 2 study *enrollment and treatment expected to be completed 1H2021***

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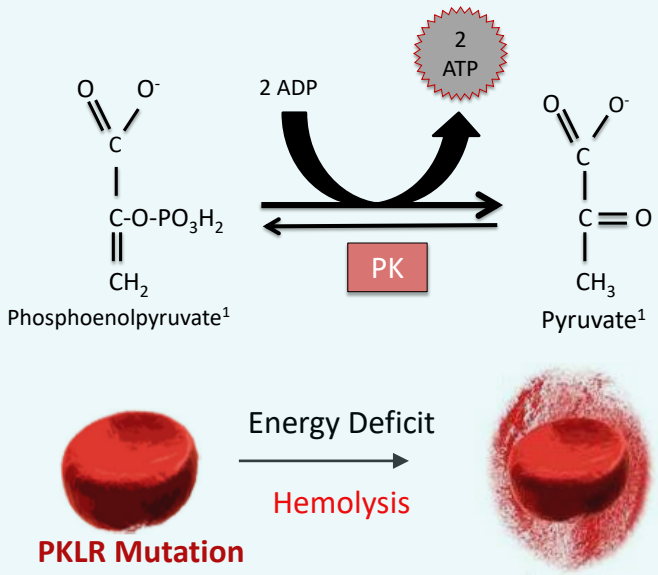
Pyruvate Kinase Deficiency (PKD)
Monogenic Red Blood Cell Hemolytic Disorder

RP-L102	RP-A501	RP-L201	**RP-L301**	RP-L401
Fanconi Anemia	Danon Disease	Leukocyte Adhesion Deficiency-I	Pyruvate Kinase Deficiency	Infantile Malignant Osteopetrosis

OVERVIEW:



- **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
- Conservative estimates conclude a number from ***3,000*** to ***8,000*** in the US + Europe combined
- **RP-L301:** ***Improvements in multiple disease components*** in a PKD mouse model, including increased hemoglobin, reduced reticulocytosis, resolved splenomegaly and reduced hepatic erythroid clusters and iron deposits

Regulatory Designations: Fast Track in the US and Orphan Drug designation in the US/EU

[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
[2]Market research indicates the application of gene therapy to broader populations could increase the annual market opportunity from approximately 250 to 500, based on an estimated prevalence in the US/EU of approximately 3,000 to 8,000.

Preclinical Studies Demonstrated Safety and Efficacy of Lentiviral-mediated Gene Therapy

PKD mice transplanted with gene-corrected cells demonstrated phenotypic correction:

- Significant increase in RBC count and half-life
- Decreased erythropoietin levels
- Normalized spleen and liver size & structure, with no evidence of erythroid clusters or iron deposits
- Improvement in red cell pyruvate kinase enzymatic pathway as assessed by metabolomic assays

Favorable Safety Results:

- No physical, behavioral biochemical, hematologic or morphologic abnormalities observed in transplanted mice
- Limited evidence of PGK-coRPK-WPRE in nonhematopoietic organs, indicating very low risk of germline transmission
- No evidence of replication competent lentivirus (RCL)

Garcia-Gomez et al. 2016; 24(7): 1187 -1198
Min-Oo, et al. Nat Gene. 2003; 35(4): 357-362.
Min-Oo, et al. Genes Immun. 2004; 5(3): 168-175.

RP-L301: Global Phase 1 PKD Gene Therapy Study

Primary Endpoint

Safety and toxicity of RP-L301

Key Secondary Endpoints

- Clinically significant reduction of anemia
- ***Transfusion independence*** (when relevant) at 12-months
- Achievement of 50% reduction in transfusion requirements (when relevant) at 12-months
- ***PB and BM*** genetic correction as demonstrated by VCN
- Reduction of hemolysis

Key Eligibility Criteria

Inclusion:

- PKD diagnosis with a confirmed *PKLR* mutation
- Age:
  - 1st cohort (N=2): ≥18 to 50-years
  - 2nd cohort (N=2): ≥12 to 17-years
  - 3rd cohort (N=2): ≥ 8 to 11-years
- ***Severe and/or transfusion-dependent anemia***
- Prior splenectomy
- Adequate cardiac, pulmonary, renal and hepatic function

Clinical Sites:

- Hospital Universitario Fundación Jiménez Díaz, Madrid
- Stanford University, Palo Alto, California
- Hospital Infantil Universitario Niño Jesús, Madrid

RP-L301: Patient Characteristics and Product Metrics

Patient Characteristics

Patient	Age (y) and Gender	Hemoglobin (g/dL)	Bilirubin (mg/dL)	Erythropoietin (mIU/mL)	Transfusion Requirement for 2 Years Prior to Enrollment
1001	31 F	7.4†	13.4 mg/dL	35.6 mIU/mL	~14 transfusion episodes
1002*	47 M	7.0‡	7.4 mg/dL	57.2 mIU/mL	~5 transfusion episodes

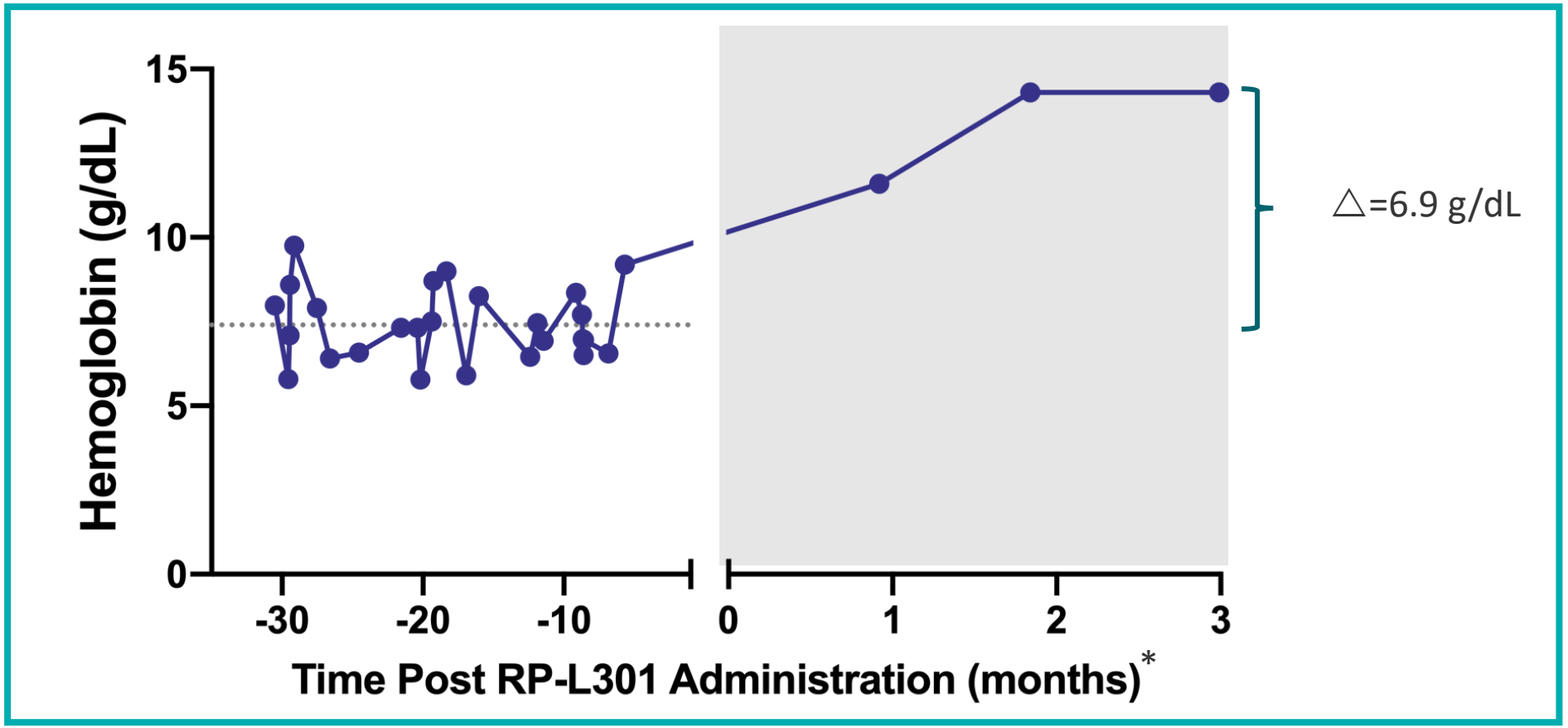
Product Metrics

Patient	CD34+ Cells/kg	Mean VCN: Liquid Culture
1001	3.9×10^6	2.73
1002*	2.4×10^6	2.08

* Infused November 2020
† Average hemoglobin calculated over 2-years prior to study enrollment
‡ Average hemoglobin calculated over 2-years prior to study enrollment; patient has declined red blood cell transfusions

Data as of October 2020

RP-L301: Preliminary Efficacy Results—L301-006-1001



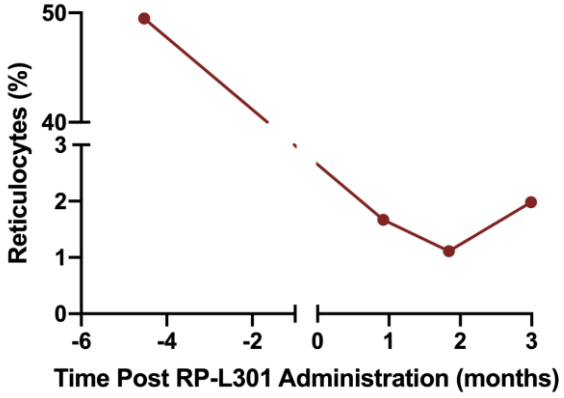
- Marked hemoglobin improvement ~7.4 g/dL to 14.3 g/dL
- No transfusion requirements following engraftment

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

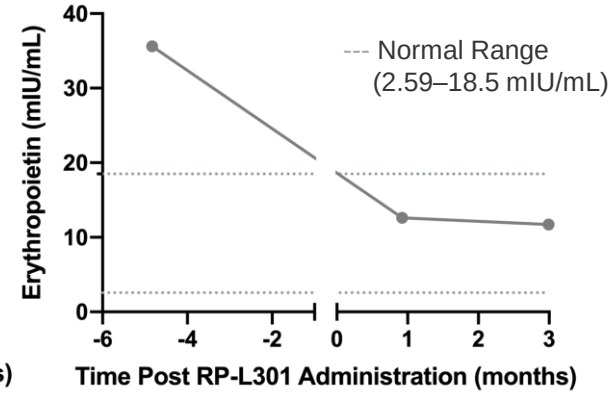
Data as of October 2020

RP-L301: Preliminary Efficacy Results—Patient 1001

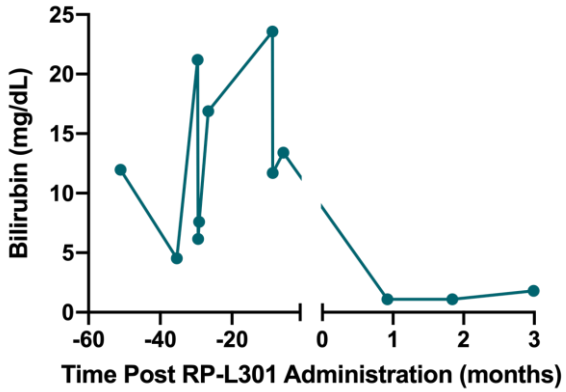
At 1–3 months post RP-L301 *



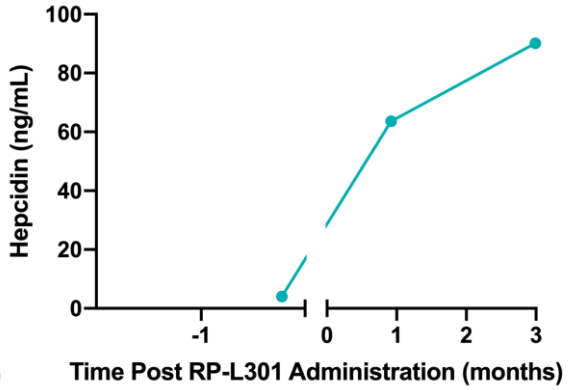
Reticulocytes decreased



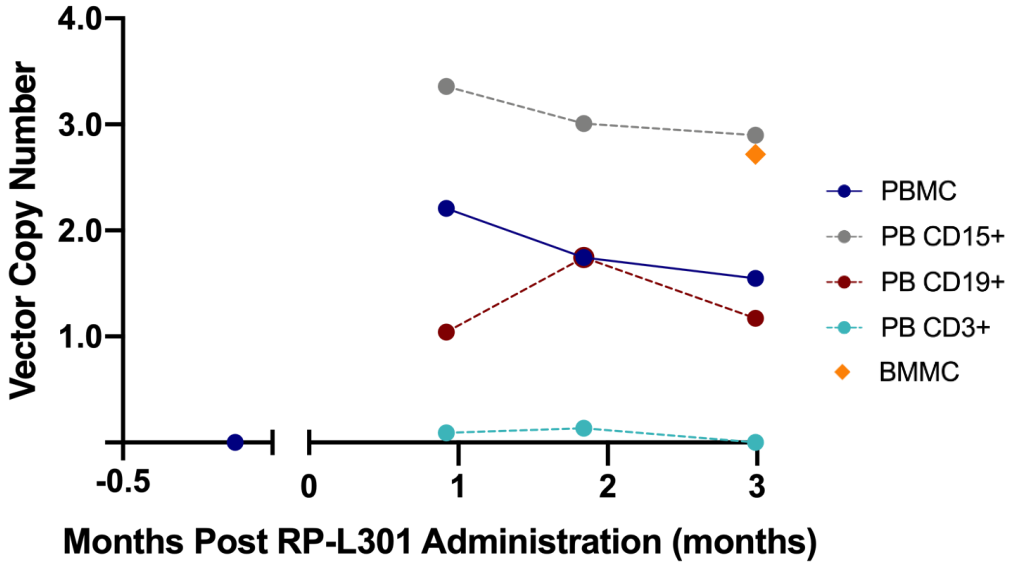
Erythropoietin normalized



Bilirubin decreased from 13.4 mg/dL to 1.8 mg/dL



Hepcidin increased from <4.0 ng/mL to 90.1 ng/mL



VCN in PBMCs 1.55 and VCN in BMMCs 2.72 at 3-months post RP-L301

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

Data as of October 2020

RP-L301: Preliminary Safety Results

Treatment-emergent Adverse Events (Grade 3 or higher) (N=1 patient)

Event	Adverse Events Grade		
System Organ Class (NCI CTCAE v. 5.0)	Any	3	4
Blood and lymphatic system disorders			
Neutropenia	1	1	–
Gastrointestinal disorders			
Stomatitis	1	1	–
Investigations			
AST increased	1	1	–
ALT increased	1	1	–
Metabolism and nutrition			
Hypertriglyceridemia	1	–	1

- ***No RP-L301 related adverse events***
- ***Patient 1001 achieved neutrophil engraftment on day +13***

Adverse events considered related to mobilization/apheresis (N=2 patients): Grade 2 SAE (chest pain, dyspnea and nausea) during apheresis collection. These events were considered related to hyperleukocytosis and the mobilizing agents. They resolved with supportive care and without sequelae. Other events included Grade 2 bone pain and Grade 3 leukocytosis.

Data as of October 2020

RP-L301 Conclusion: Hemoglobin Normalized in First Patient

- Safety profile of RP-L301 ***appears favorable***
  - Infusion well tolerated (n=1); no IP-related SAEs or AEs
  - Hematopoietic reconstitution in less than 2-weeks in initial patient
- Preliminary efficacy activity observed during initial 3-months after administration of RP-L301
  - Patient 1001 with peripheral blood VCN of 1.55 at 3-months, ***hemoglobin nearly doubled*** and normalized hemolysis markers (Hb from baseline ***increased ~7g/dL*** at 3-months post RP-L301)
- Second cohort will enroll older pediatric patients and is expected to be initiated in 1H2021

Commercial-grade drug product and centralized testing for all treated patients

Data as of October 2020

Infantile Malignant Osteopetrosis (IMO)
Monogenic bone resorption disorder

RP-L102
Fanconi Anemia

RP-A501
Danon Disease

RP-L201
Leukocyte Adhesion Deficiency-I

RP-L301
Pyruvate Kinase Deficiency

RP-L401
Infantile Malignant Osteopetrosis

OVERVIEW:

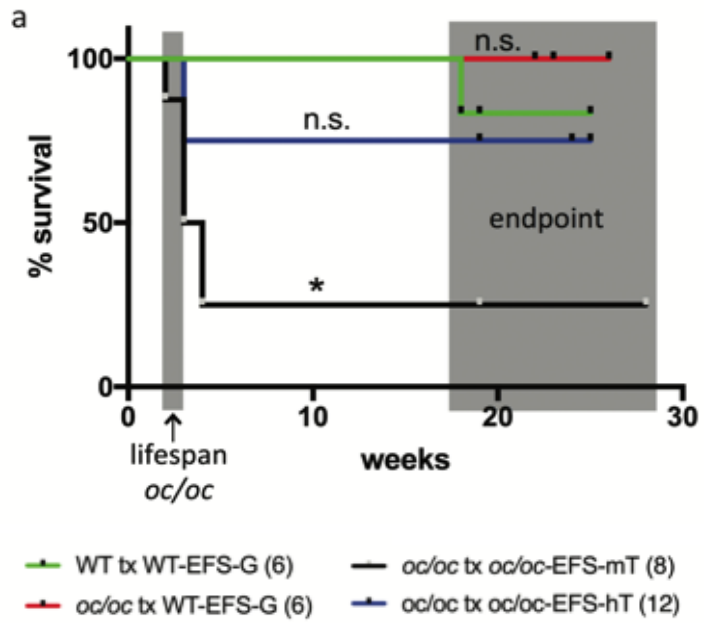
- **Background:** ***Dysfunctional osteoclast*** disease characterized by bone marrow failure, skeletal deformities, and neurologic abnormalities caused by *TCIRG1* mutations in >50% of cases[1]
  - ***Frequent mortality in early years of life, severe marrow failure and visual impairment during 1st year***
- **Current Available Treatments:** Hematopoietic stem cell transplants associated with GVHD and ***limited efficacy***
- **Addressable Market**: ***>50*** patients/year[2]
- **RP-L401:** *In vitro* restoration of osteoclast resorptive function observed; *in vivo* correction in murine model
- **Regulatory Designations:** Rare Pediatric Disease, Orphan Drug and Fast Track designations in the US

[1]Source: https://www.orpha.net/consor/cgi-bin/OC_Exp.php?lng=EN&Expert=667
[2]Estimated incidence of one in 200,000 live births; Source: http://www.orpha.net/consor/cgi-bin/OC_Exp.php?lng=EN&Expert=667

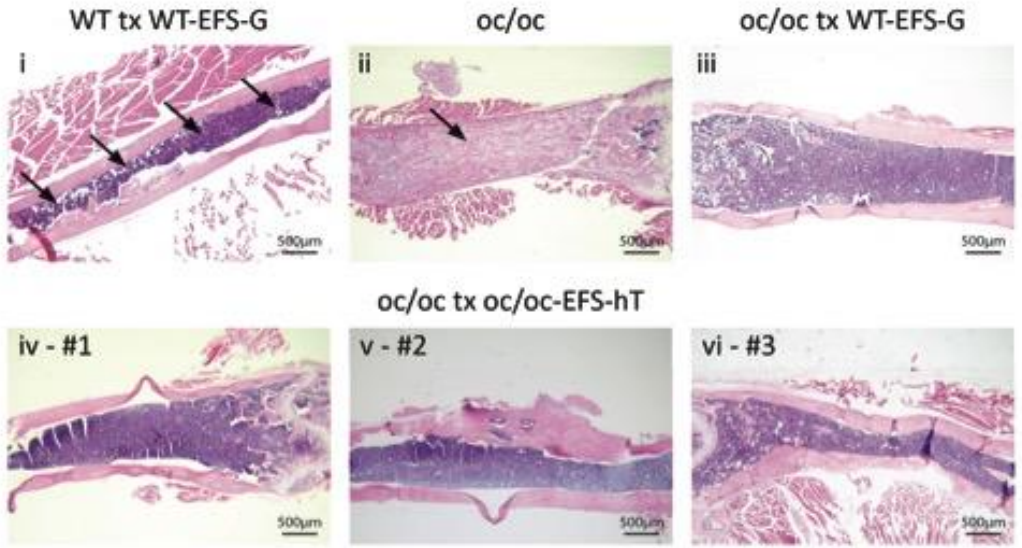
Preclinical Mouse Data Supports Progression to Phase 1

Oc/oc mice receiving RP-L401 showed correction of the disease phenotype, with increased long-term survival, tooth eruption, weight gain, and normalized bone resorption

Increased Long-term Survival



Reversal of Osteopetrotic Bone Phenotype



E-Poster Presented at ESID October 2020

RP-L401 (IMO) Clinical Trial and Outcome Measures[1]

Non-Randomized Phase 1 Study

Design

- Enroll 2 patients, with a confirmed diagnosis of IMO with documented *TCIRG1* mutation
  - 1-month or older

Primary Outcomes

- Safety associated with treatment

Secondary Outcomes

- Normalization of serum calcium and blood counts
- Presence of gene-modified blood and bone marrow cells
- Normalization of bone abnormalities on X-ray and DEXA scans
- Prevention or stabilization of vision and hearing loss
- Reduction in hepatosplenomegaly

[1]Source: https://www.clinicaltrials.gov/ct2/show/NCT04525352?term=NCT04525352&draw=2&rank=1

rocket pharma

Growing IP Portfolio

Multiple in-licensed patent families for GTx products and related technology platforms

Supporting current pipeline efforts:

- Four In-licensed pending international patent applications filed under Patent Cooperation Treaty (PCT):
  - FA (2)
  - LAD-I
  - PKD
- Multiple patent applications pending:
  - Danon (exclusive world-wide license from UCSD)
- Multiple patent families licensed from REGENXBIO:
  - Danon – AAV9 (exclusive world-wide license)
  - Danon – 2 undisclosed capsid serotypes (exclusive world-wide option to license)
- Multiple cell and gene therapy platform technologies licensed for pipeline product improvements

Rocket Proprietary Filed IP

Extensive patent portfolio across multiple platforms:

- Multiple pending patent applications for ex-vivo LVV programs
- Multiple pending patent applications for in-vivo AAV

World-Class Research and Development Partners

CIBER	IIS FJD	REGENXBIO	University of California, Los Angeles
CIEMAT	Lund University	Stanford Medical School	University of Minnesota
Fred Hutchinson Cancer Research Center	Memorial Sloan Kettering Cancer Center	UCL	University of Pennsylvania
Hospital Universitario Fundación Jiménez Díaz	Niño Jesús Hospital	University of California, San Diego	

Expansion into Cranbury, NJ: R&D/CMC Efforts and Eventual cGMP Manufacturing

2021

- Continue R&D to ***further support*** CMC analytics and internal QC and release testing activities for RP-A501
- 50,000 sq. ft. from this facility will be ***dedicated to AAV cGMP*** manufacturing (FDA and EMA compliant)
- Initiate in-house GMP clinical manufacturing
- Enables ***dual-sourcing*** for Danon commercial capacity

RCKT Cranbury (NJ)
103,720 sq. ft. production facility

Near and Long-Term Value Drivers

Potential for Five Gene Therapy Products to be Approved by 2025