ASH 2020: Investor & Analyst Event

December 7, 2020

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SEEKING GENE THERAPY CURES

Participants

Gaurav Shah, MD
Chief Executive Officer and President, Rocket Pharmaceuticals

Kinnari Patel, PharmD, MBA
Chief Operating Officer & Head of Development, EVP

Jonathan Schwartz, MD
Chief Medical Officer & Clinical Development, SVP

Claudine Prowse, PhD
SVP, Strategy & Corporate Development

Gayatri Rao, MD, JD
VP, Global Program Head, LVV

Maria Grazia Roncarolo, MD
Professor in Stem Cell and Regenerative Medicine; Professor of Pediatrics, Medicine; Co-Director of the Institute for Stem Cell Biology and Regenerative Medicine; Director of the Center for Definitive and Curative Medicine at the Stanford University School of Medicine

Agnieszka Czechowicz, MD, PhD
Assistant Professor of Pediatrics, Division of Stem Cell Transplantation and Regenerative Medicine at the Stanford University School of Medicine (FA)

Rachael Grace, MD, MMSc
Director, Hematology Clinic; Assistant Professor of Pediatrics, Harvard Medical School

Agenda

1. Opening Remarks
   - Claudine Prowse, PhD—SVP, Strategy & Corporate Development
2. RP-L102: Fanconi Anemia
   - Gaurav Shah, MD—Chief Executive Officer & President
3. RP-L301: Pyruvate Kinase Deficiency
   - Jonathan Schwartz, MD—Chief Medical Officer & Clinical Development, SVP
4. RP-L201: Leukocyte Adhesion Deficiency-I
   - Kinnari Patel, PharmD, MBA—Chief Operating Officer & Head of Development, EVP
5. Q&A Session

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 the safety, effectiveness and timing of product candidates that Rocket may develop, including in collaboration with academic partners, 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 patient 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 successfully demonstrate the efficacy and safety of such products and pre-clinical studies and clinical trials, its gene therapy programs, the preclinical and clinical results for its product candidates, which may not support further development and marketing approval, the potential advantages of Rocket's product candidates, 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 and its licensors ability to obtain, maintain and protect its and their respective intellectual property, the timing, cost or other aspects of a potential commercial launch of Rocket's product candidates, Rocket's ability to manage operating expenses, Rocket's ability to obtain additional funding to support its business activities and establish and maintain strategic business alliances and new business initiatives, 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 Securities and Exchange Commission. 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.

RP-L102: Fanconi Anemia

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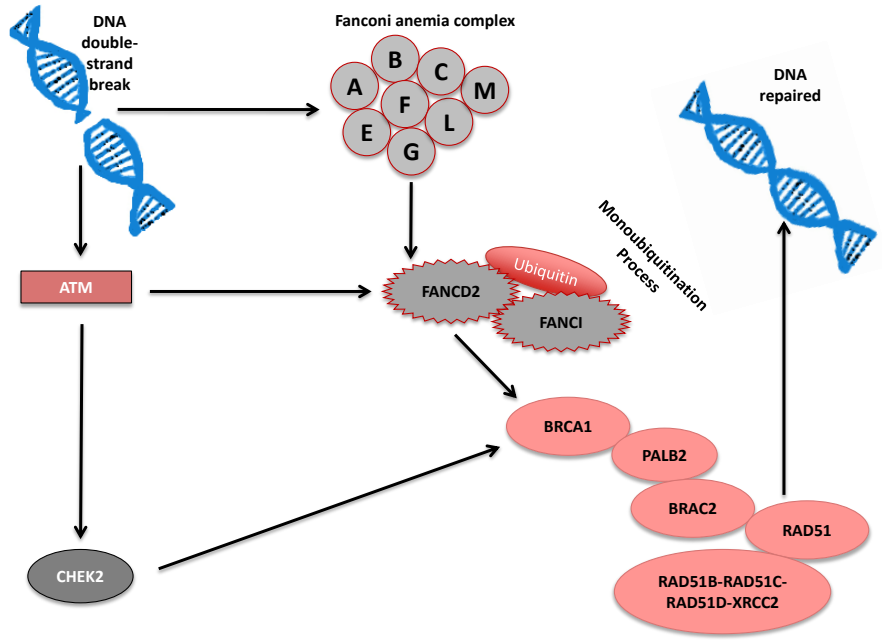
What is Fanconi Anemia (FA)?

- **FA**: a rare genetic DNA repair disorder
- **Background:** mutations in FANC family of genes which are involved in DNA repair
  - FANCA mutations: 60-70% of cases
- **Disease Sequelae:** Birth defects, developmental issues, ***80% bone marrow failure by age 10***, acute myeloid leukemia and head and neck cancer risk increase by 30-50x[1]
- **Current Available Treatments:** Allogeneic HSC transplant associated with 100-day mortality, GVHD and additional increased cancer risk
- **Addressable Market[2]:** ~4,000 patients; ~500 patients/year (U.S. & Europe)



[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

FANCOLEN-I: Proof of Concept & Key Takeaways

- Study utilized academic ***"Process A"***
- 9 pediatric patients[1] have been treated (enrollment complete)
- Patients had varying degrees of bone marrow health at baseline and received a wide range of drug product CD34+ cell numbers and VCN

Process A Takeaways:[1]

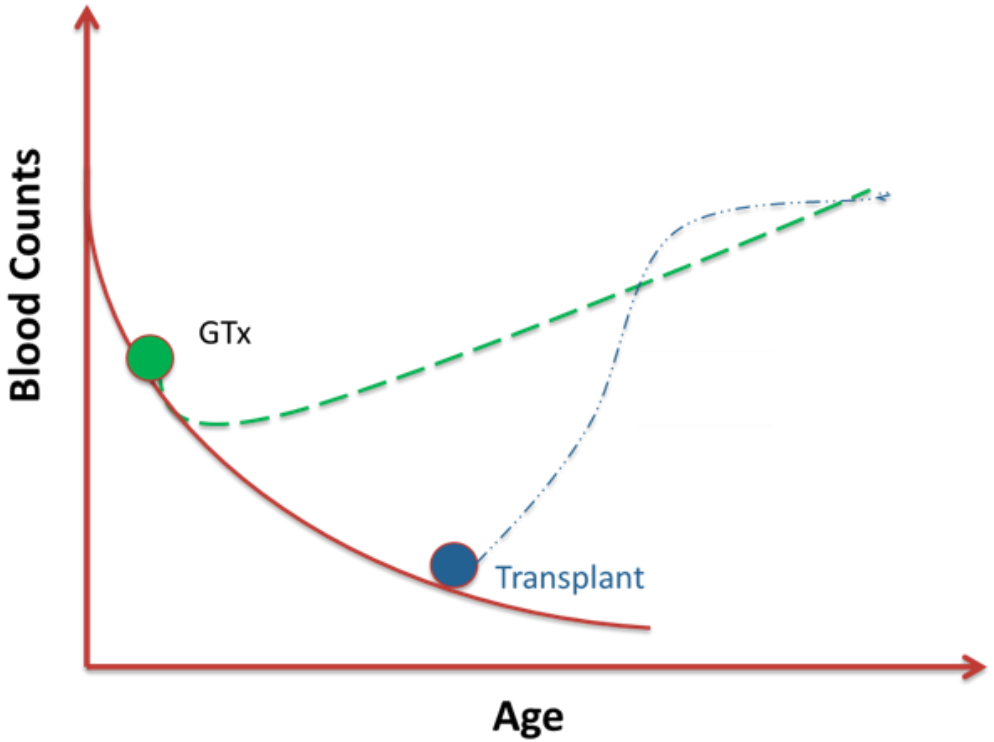
- Favorable engraftment results without conditioning due to selective advantage of gene corrected cells
  - Without conditioning initial engraftment observed between 1-3 years post-treatment
- Engraftment is correlated with stabilization and/or clinical improvement in blood counts
- Results led to PRIME, RMAT, ATMP, Fast Track, Rare Pediatric & Orphan Drug (US/EU) Designations

1 Four of these patients have been followed for more than 2 years (24-39 months for patients 02002, 02004, 02005, and 02006)

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

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.



RP-L102 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

Median values:
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.

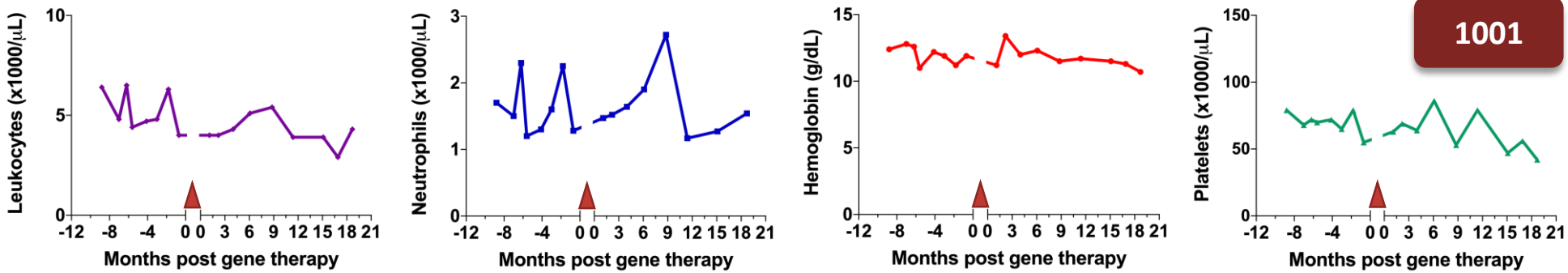
^ Per NC200 automated count (results in ~50% lower count vs. manual used in FANCOLEN-I).

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

Data as of October 2020

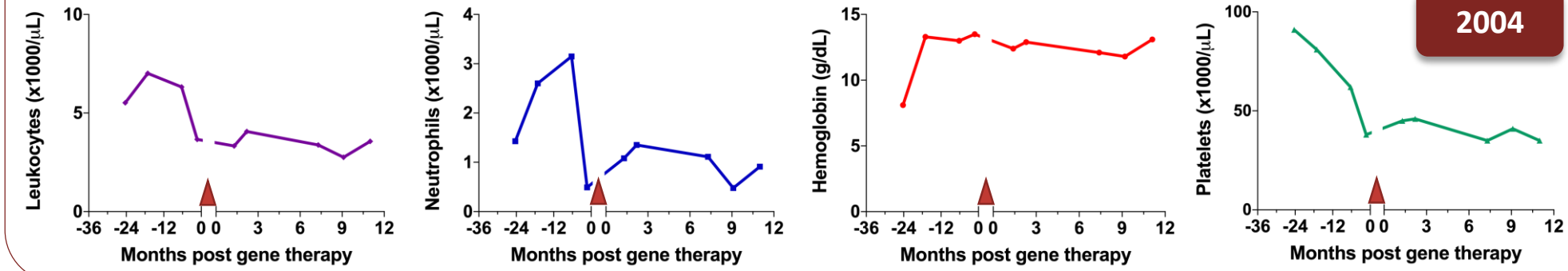
Optimal means of the nine patients treated in Fancolen-I the two that had the best benefit risk

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



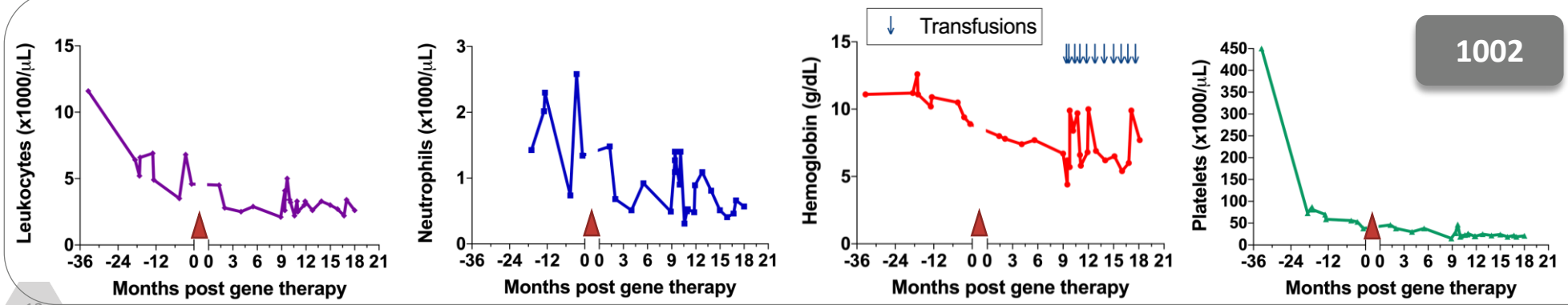
1001

- 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



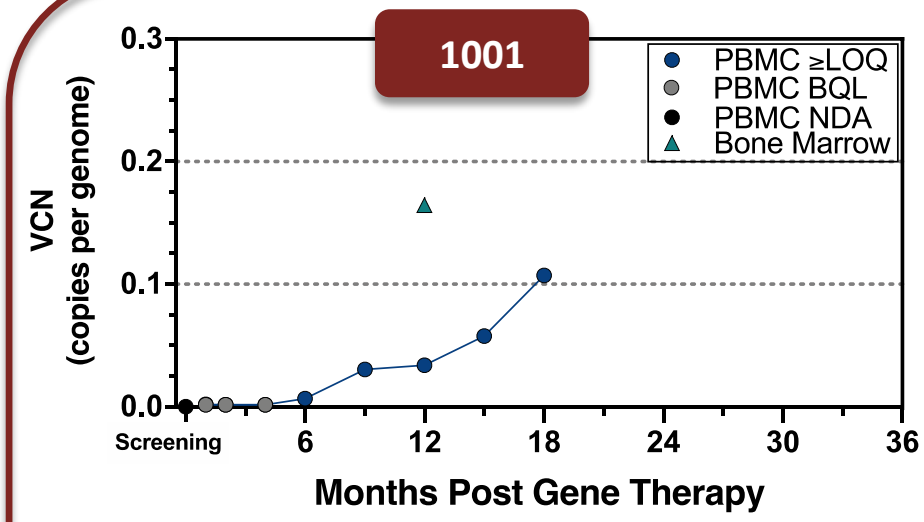
1002

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

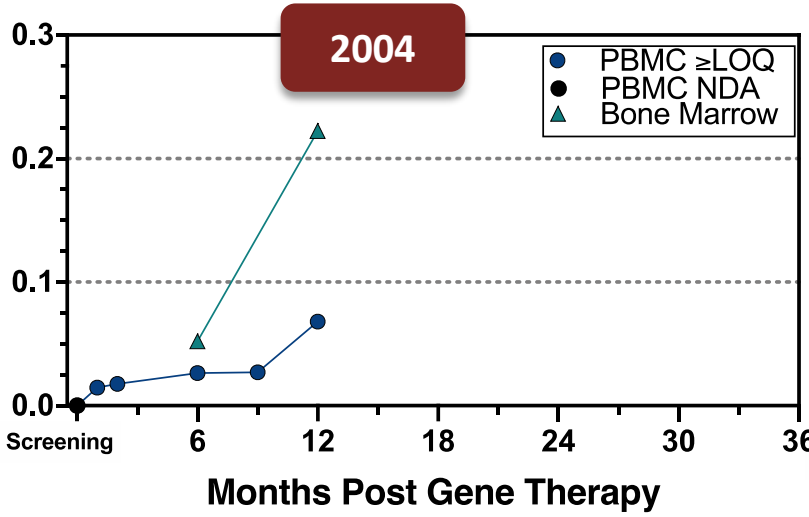
RP-L102 Treated Study Patients (>12M Follow-up)

N = 3 with ≥ 12-Months of Follow-up

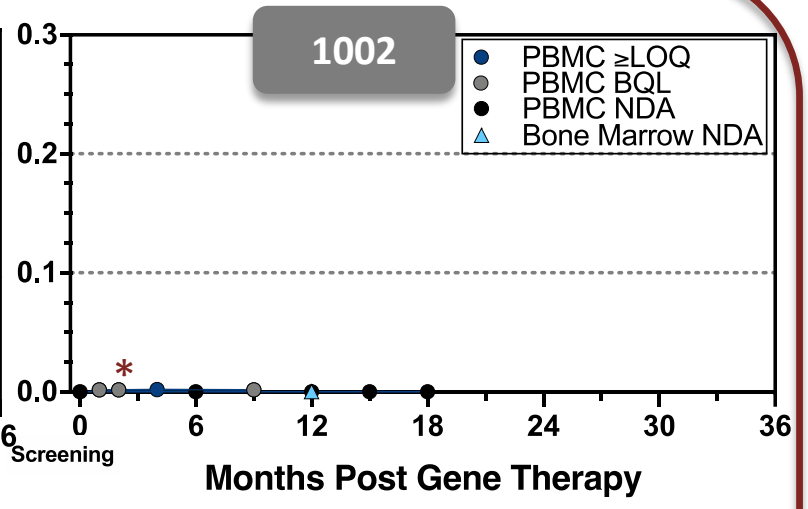
- 2 of 3 showed increasing evidence of engraftment
- 1 patient's course (1002) complicated by *Influenza B* infection; received BMT
- *Of note: Additional 3 of 4 patients with 2-months follow-up have early evidence of engraftment (0.01-0.02)*



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



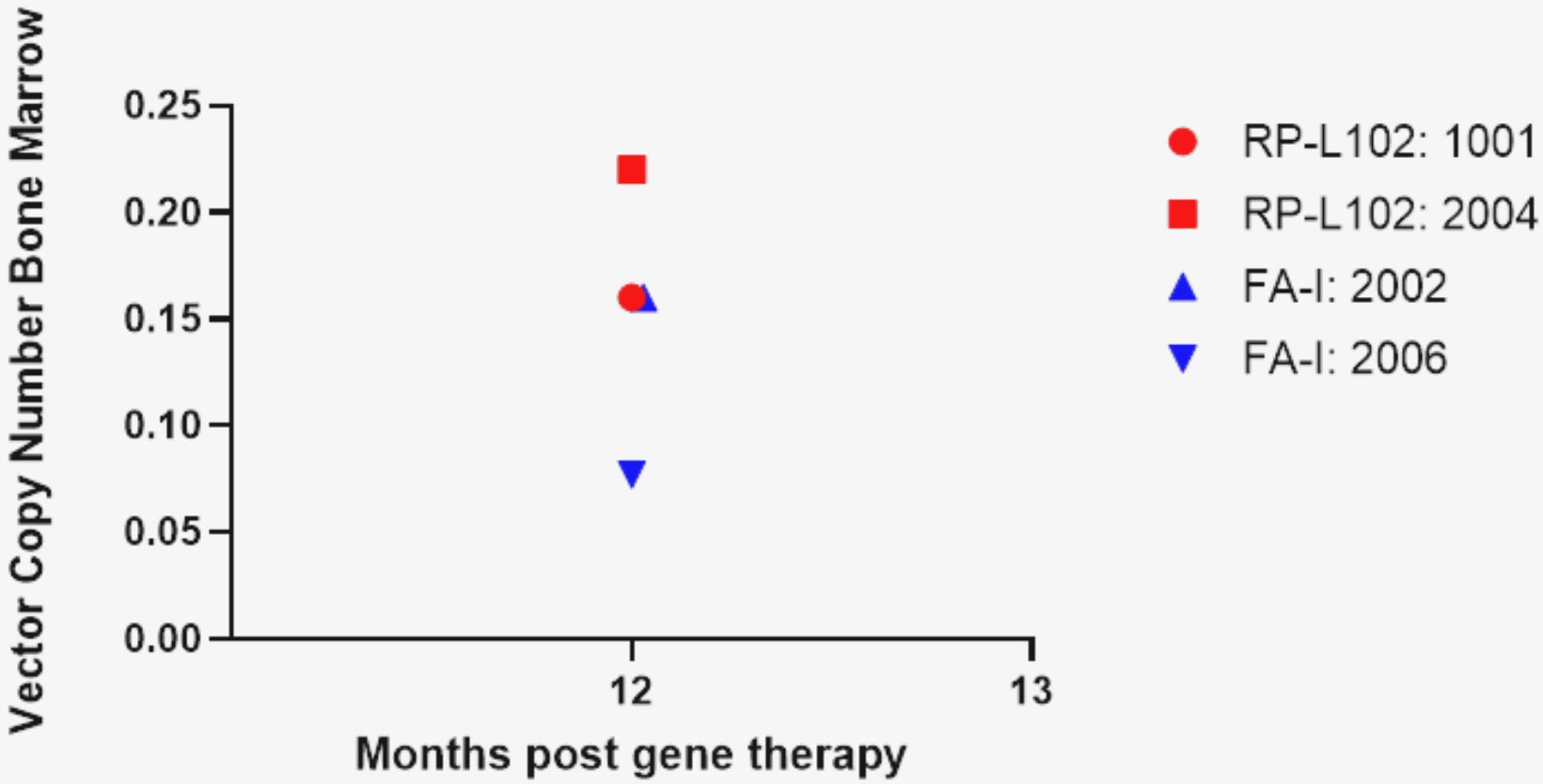
BM progenitors resistant to 10 nM MMC at 12-months suggests engraftment, but with insufficient rapidity to prevent influenza/FA-mediated BMF

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)

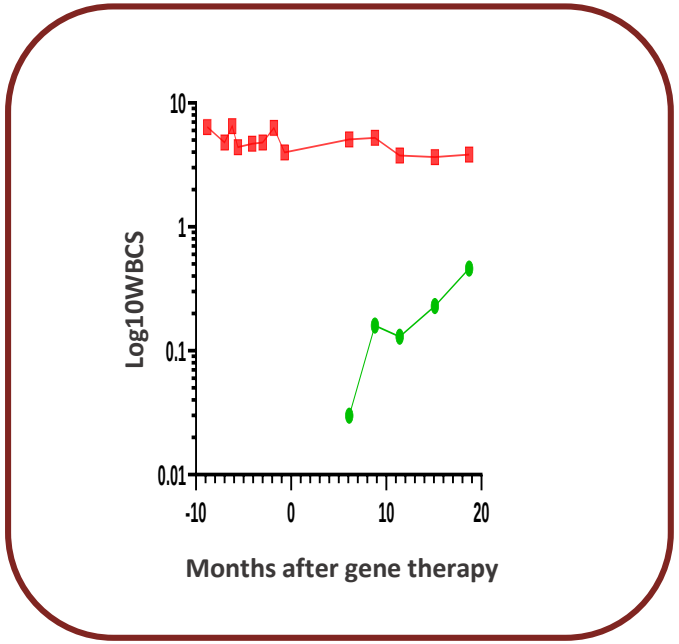
"Process B" BM VCN was in Line or Better than Optimally Treated "Process A" Patients at 12-Months



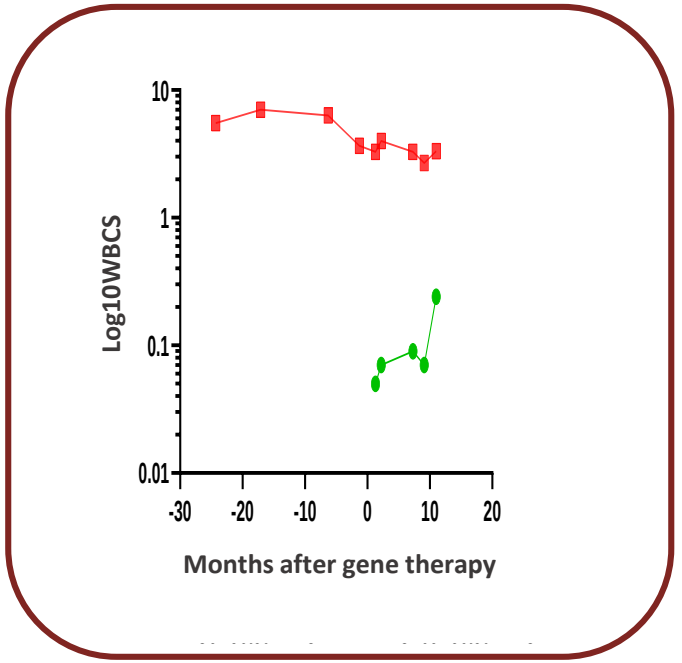
Optimal means of the nine patients treated in Fancolen-I the two that had the best benefit risk

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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RP-L301: Pyruvate Kinase Deficiency

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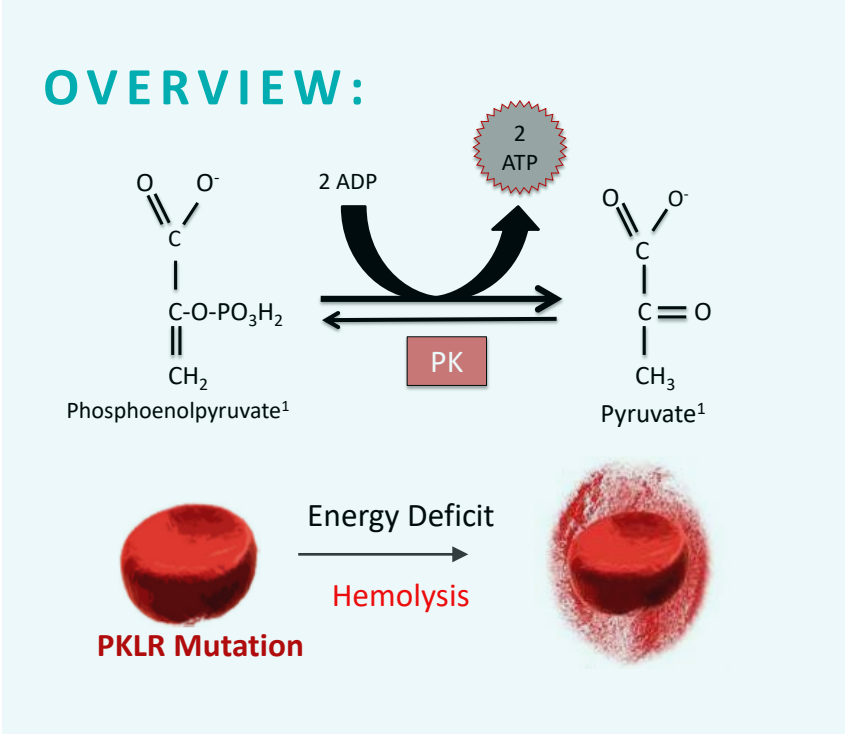
What is Pyruvate Kinase Deficiency (PKD)?

- **PKD**: rare inherited hemolytic anemia, caused by *PKLR* gene mutation
- **Background:** results in decreased red cell pyruvate kinase activity, impaired erythrocyte metabolism and diminished red blood cell lifespan.
  - Characterized by anemia, reticulocytosis, hyperbilirubinemia, splenomegaly and iron overload—may be life threatening in severely affected individuals
- **Current Available Treatments:** chronic blood transfusions, iron chelation therapy and splenectomy
  - Side effects: increased infection susceptibility and thromboembolic risk
  - Therapies do not ameliorate PKD-related iron overload or end-organ damage.
- **Addressable Market[1]:** ~250-500 patients/year



1 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.

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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***
- 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

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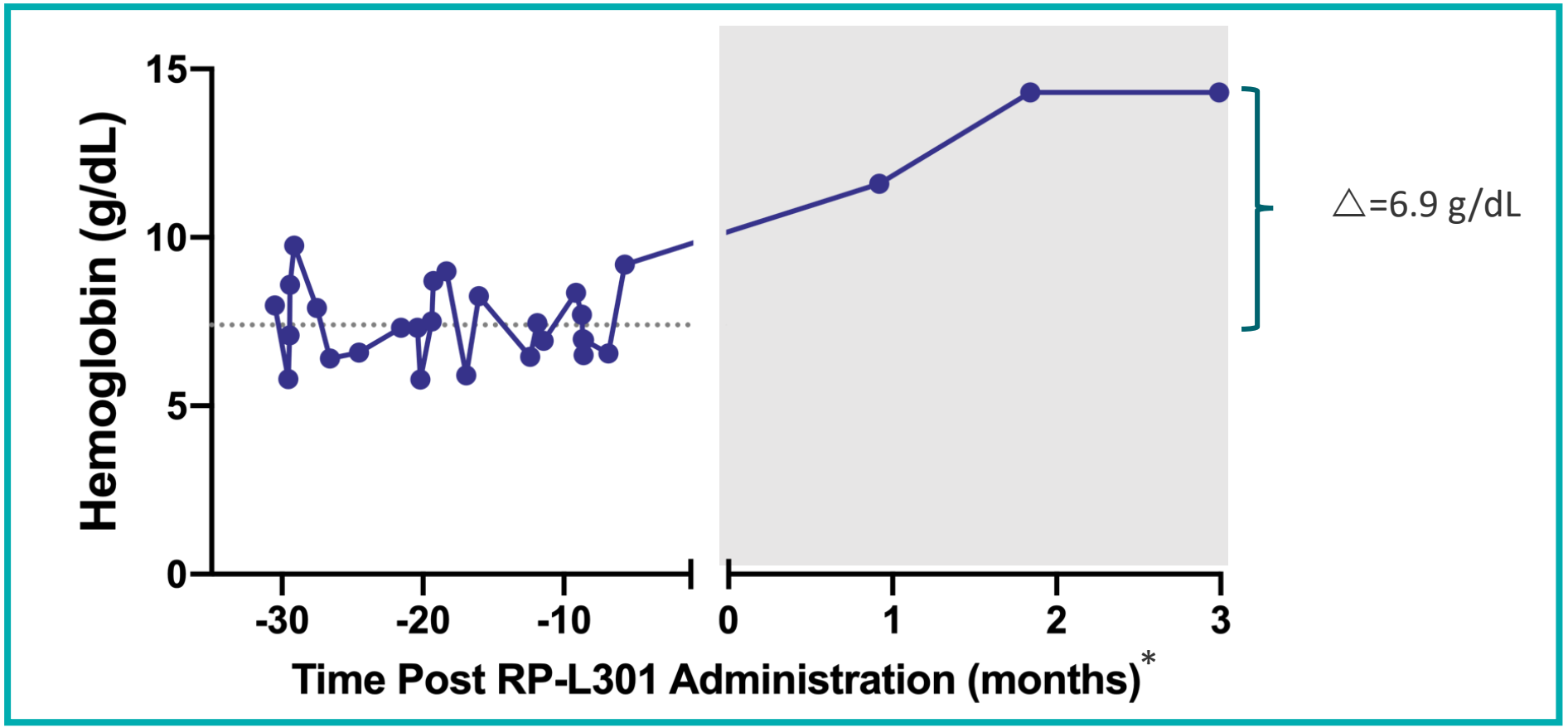
RP-L301: Preliminary Efficacy Results—Patient 1001



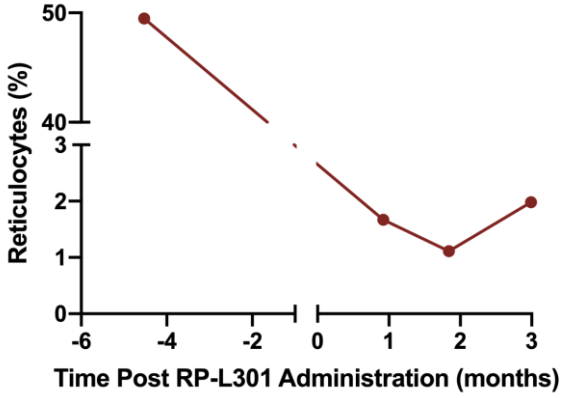
- Marked hemoglobin improvement ~7.4 g/dL to 14.3 g/dL
- No transfusion requirements following engraftment

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

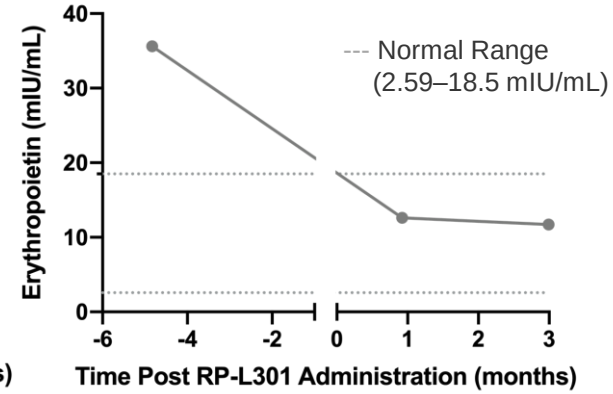
Data as of October 2020

RP-L301: Preliminary Efficacy Results—Patient 1001

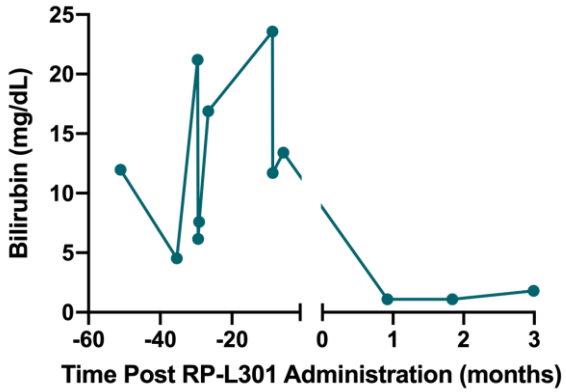
At 1–3 months post RP-L301 *



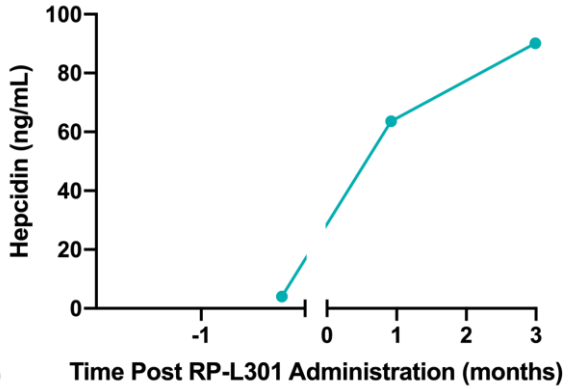
Reticulocytes decreased



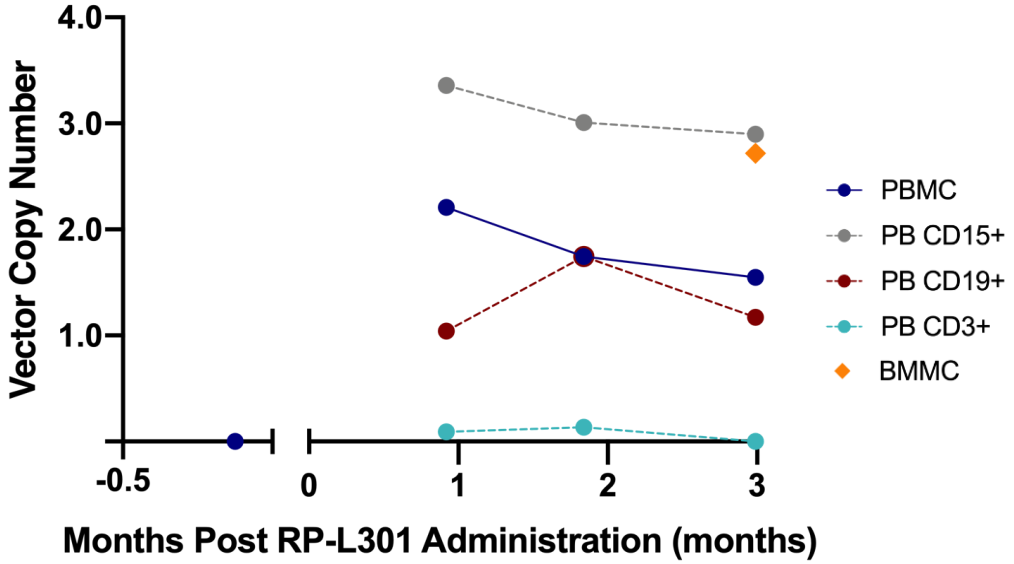
Erythropoietin normalized



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

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RP-L201: Leukocyte Adhesion Deficiency-I

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LAD is CIRM funded

What is Leukocyte Adhesion Deficiency-I (LAD-I)?

- Mutations in the *ITGB2* gene encoding for β2-integrin common chain (CD18)
  - Prevents expression of functional CD18/CD11 heterodimers on WBC cell surface
  - Leukocytes unable to leave bloodstream and migrate to sites of infection
- Characterized by ***recurring*** and ultimately ***fatal infections***
- Current Treatment Option: Allogeneic HSCT—frequently limited by donor availability, frequent graft rejection & acute GvHD, infections
- Addressable Market: 25-50 pts/yr; up to 100 for potential expansion into moderate population in the US + Europe

Clinical Pathogenesis of LAD-I

LAD-I Disease Spectrum

Severe:
<2%
CD18+ PMN

Moderate:
2-30%
CD18+ PMN

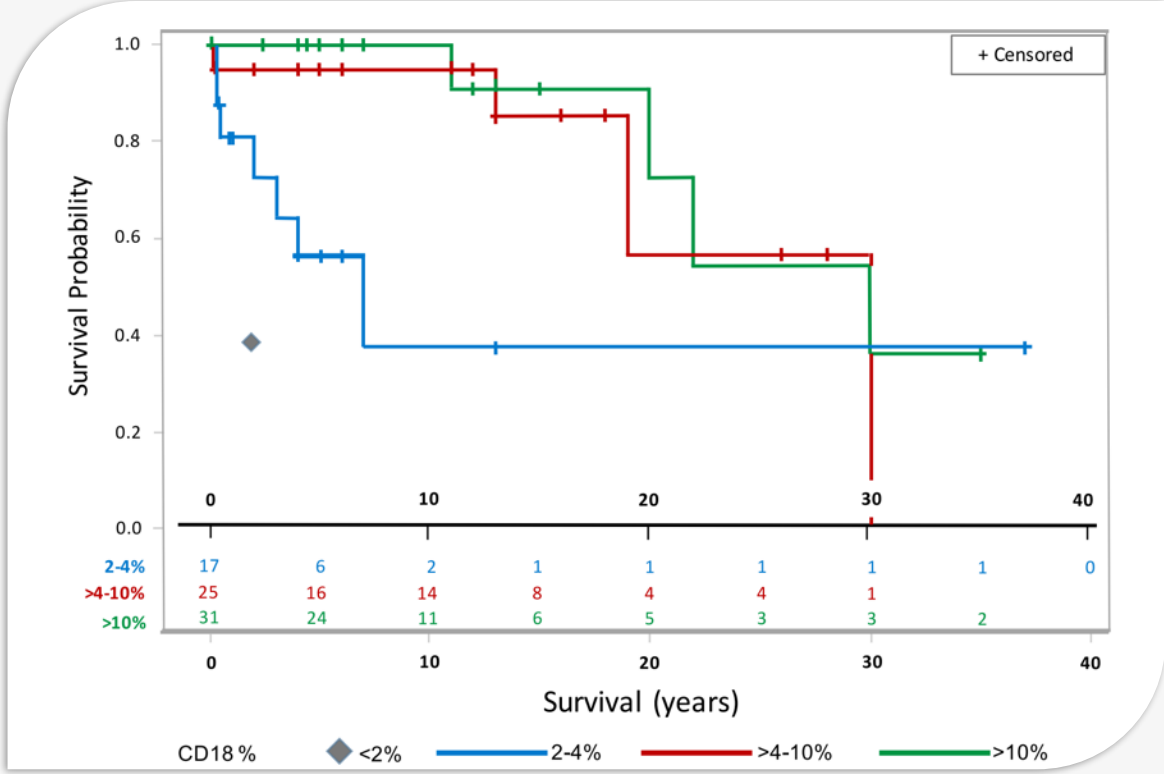
PMN = polymorphonuclear neutrophils

LAD-I Clinical Prognosis

- Patients suffer from recurrent infections; fatal in majority
  - >50% patients with severe variant
    - 60-75% with severe LAD-I die **prior to age 2**
  - >50% with moderate LAD-I die **before age 40**

Kaplan-Meier Survival Estimates by Neutrophil CD18 Expression

-Patients with LAD-I not receiving allogeneic HSCT-



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

1 Source: Almarza Novoa E et al. J Allergy Clin Immunol Pract. 2018 Jan 20. pii: S2213-2198(17)31026-7

RP-L201 Clinical Trial Design and Status

Adaptive Phase 1/2 Study

Design

Phase	N (Planned)	DP Manufactured	N (Treated)
1	2	2	2
2	7	5	2
Total	9	7	4

Primary Outcomes

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

Key Secondary Outcomes

- % of pts w/neutrophil CD18 expression at least 10% of normal
- Resolution (partial or complete) of underlying skin rash or periodontal abnormalities

Data from N=3 patients described | N=1 patient recently treated

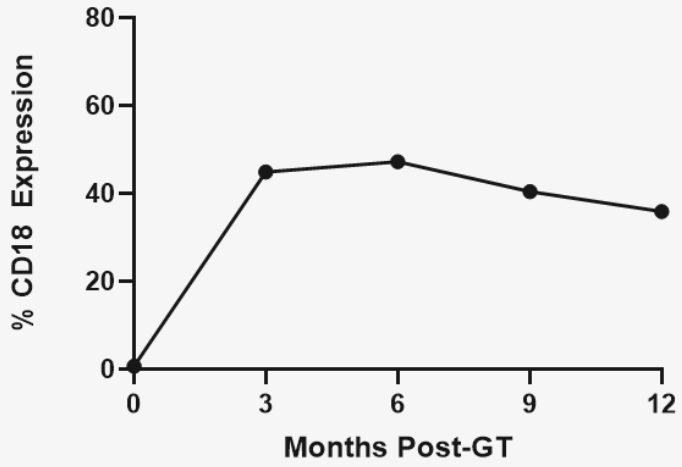
Patient 1001: 12-Month Follow-Up

9-y.o. female diagnosed with severe LAD-I at age 7

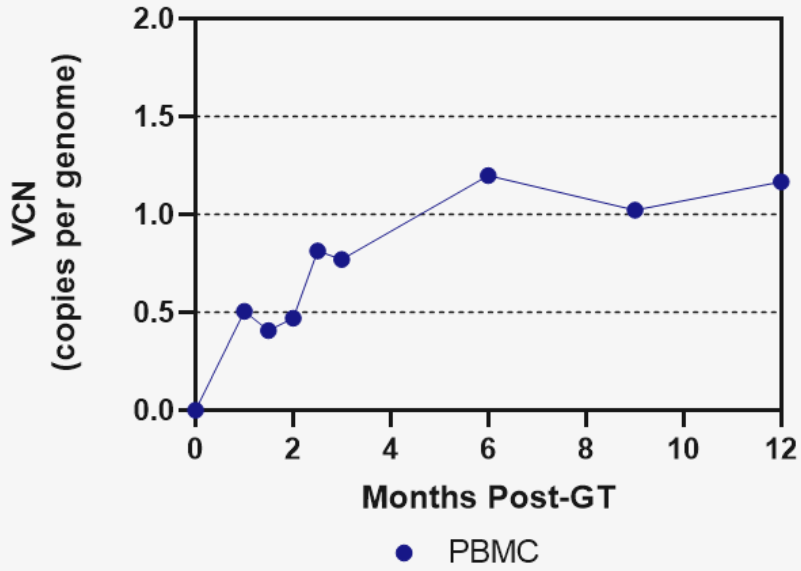
Key Drug Product Metrics

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

% CD18 Expression (PMN)



VCN (PBMC)



PMN: polymorphonuclear lymphocytes

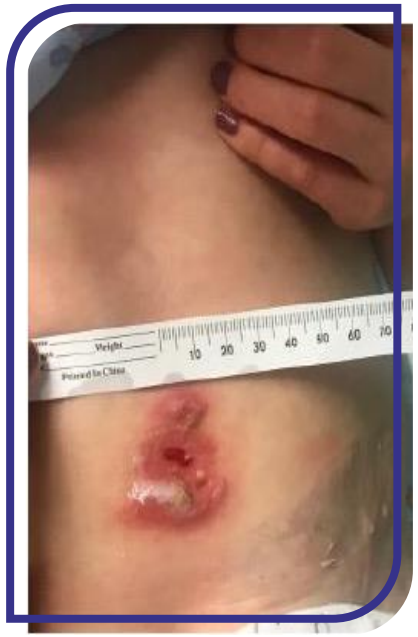
PBMC: peripheral blood mononuclear cell

Patient 1001: Visible Improvements Post-Treatment

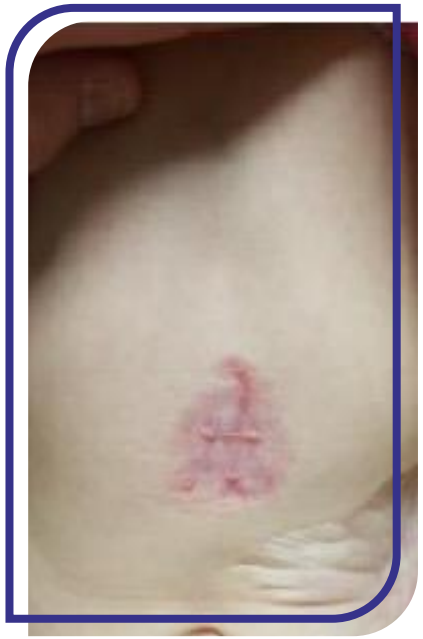
Pre GTx: Severe infections ≥ 1 per year; numerous hospitalizations, severe skin lesions, continuous prophylactic antibiotics and required home schooling

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

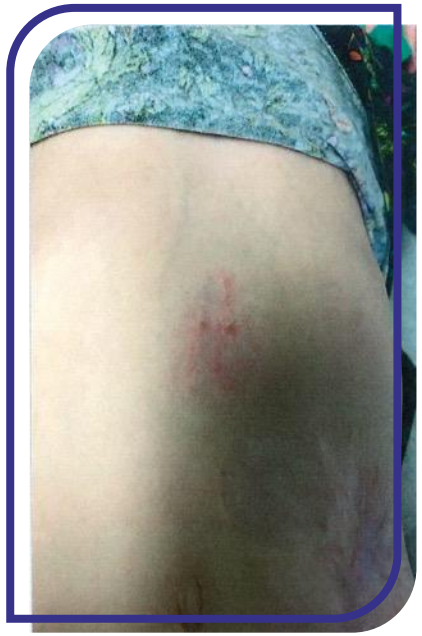
Spontaneous Abdominal Lesion (pyoderma gangrenosum)



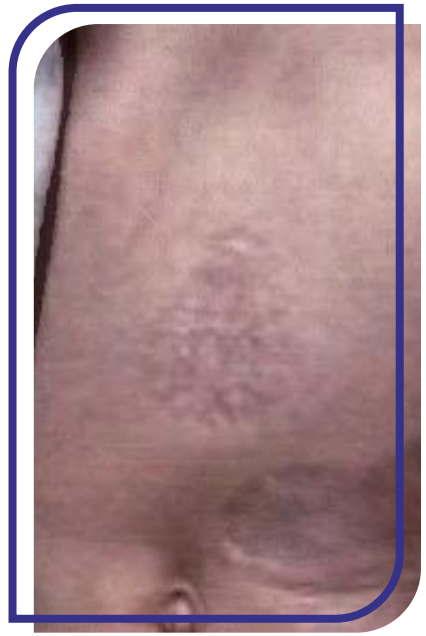
Baseline (Pre-Treatment)



3-months (Post-Treatment)



6-months (Post-Treatment)



12-months (Post-Treatment)

UCLA Mattel Children's Hospital Data

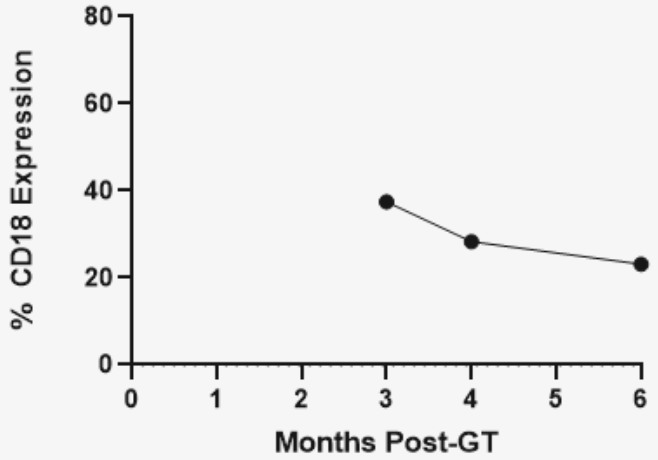
Patient 1004: 6-Month Follow-Up

3-y.o. female diagnosed with severe LAD-I at age 3

Key Drug Product Metrics

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

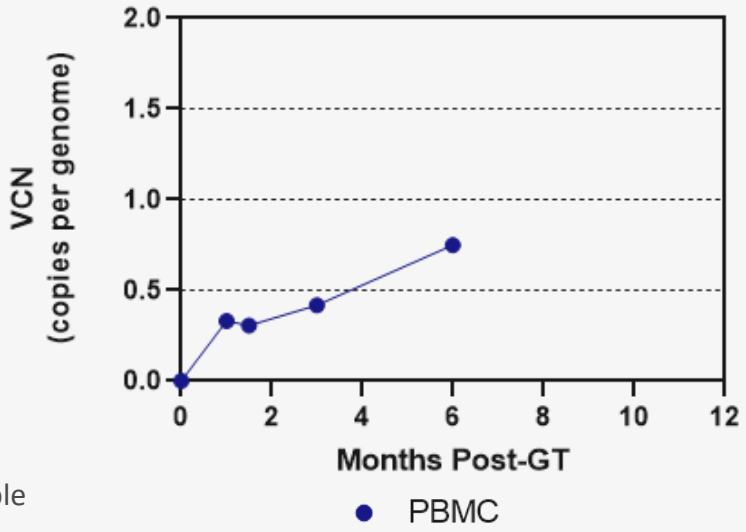
% CD18 Expression (PMN)



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

Patient 2006: 2-Month Follow-up

7-m.o. male diagnosed at birth

KEY DRUG PRODUCT METRICS

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

Drug Product VCN:
2.87

Hematopoietic reconstitution observed post-infusion

76% CD18 expression at 2-month timepoint

Clinically stable with no reported serious adverse events post-infusion

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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THANK YOU!

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Question & Answer Panelists

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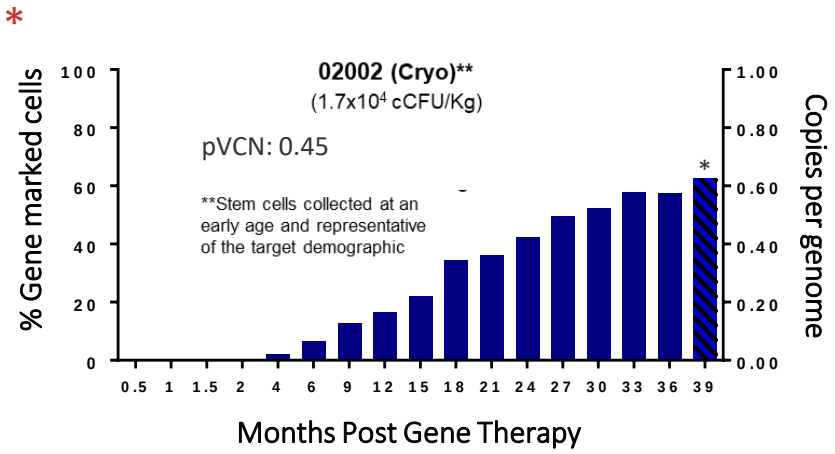
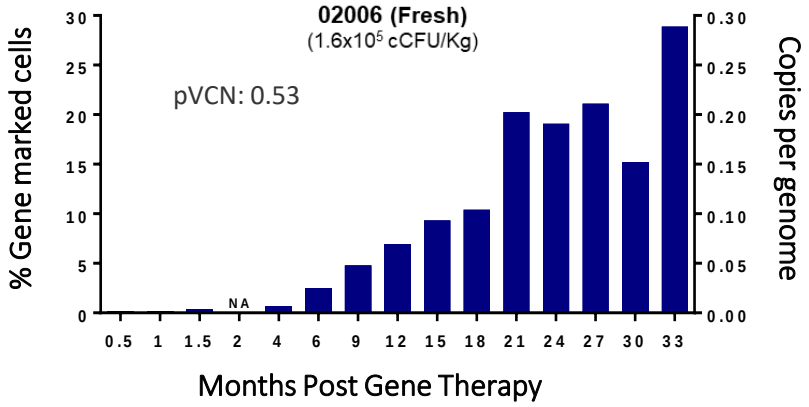
Appendix

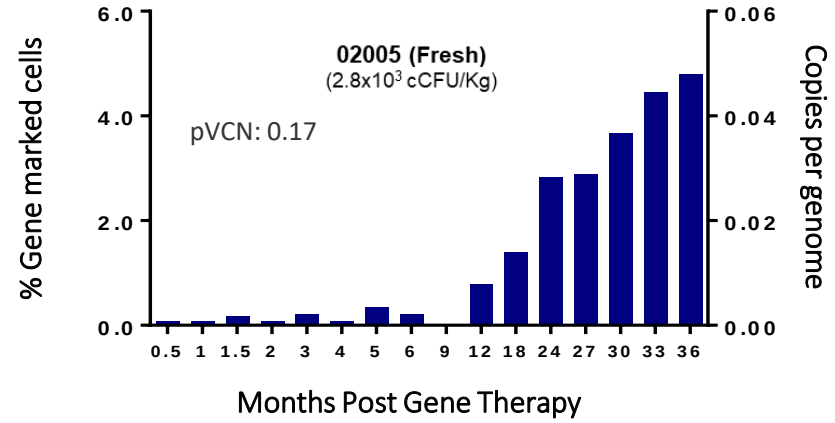
Process A: Bone Marrow Engraftment

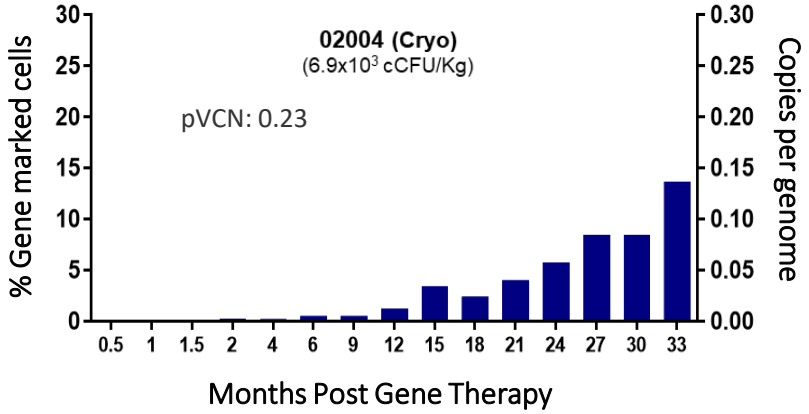
First Demonstration of Engraftment Without Conditioning ("Process A"—non-optimized—RP-L102)

pVCN: Product VCN









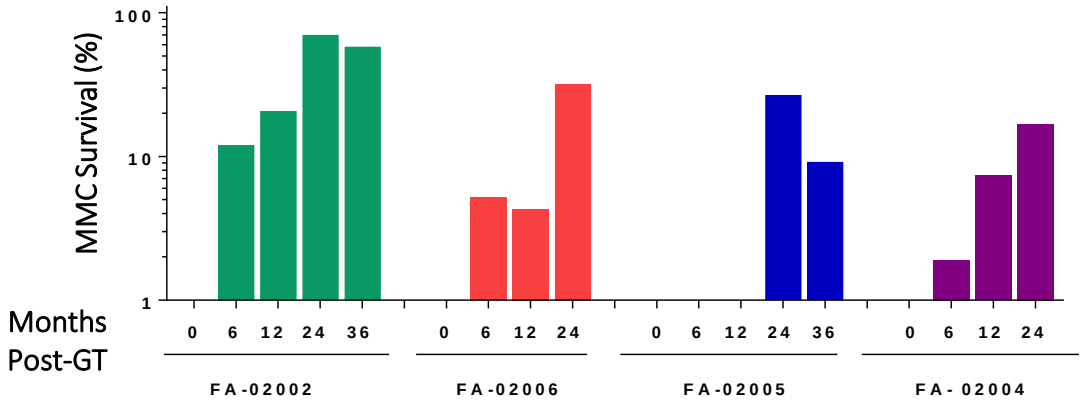
HIUNJ Data Presented at ASGCT By CIEMAT May 2020

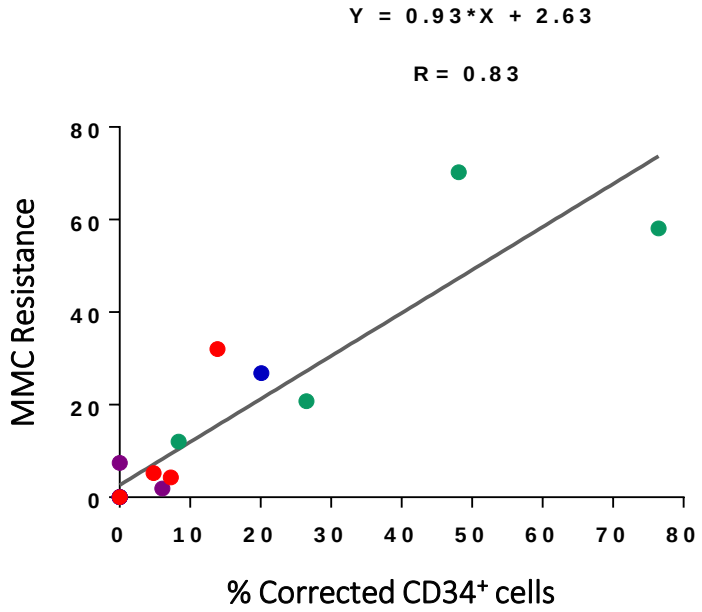
cCFU = Corrected Colony Forming Units; pVCN: Product VCN *Minimally Acceptable Dose

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

Process A: 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

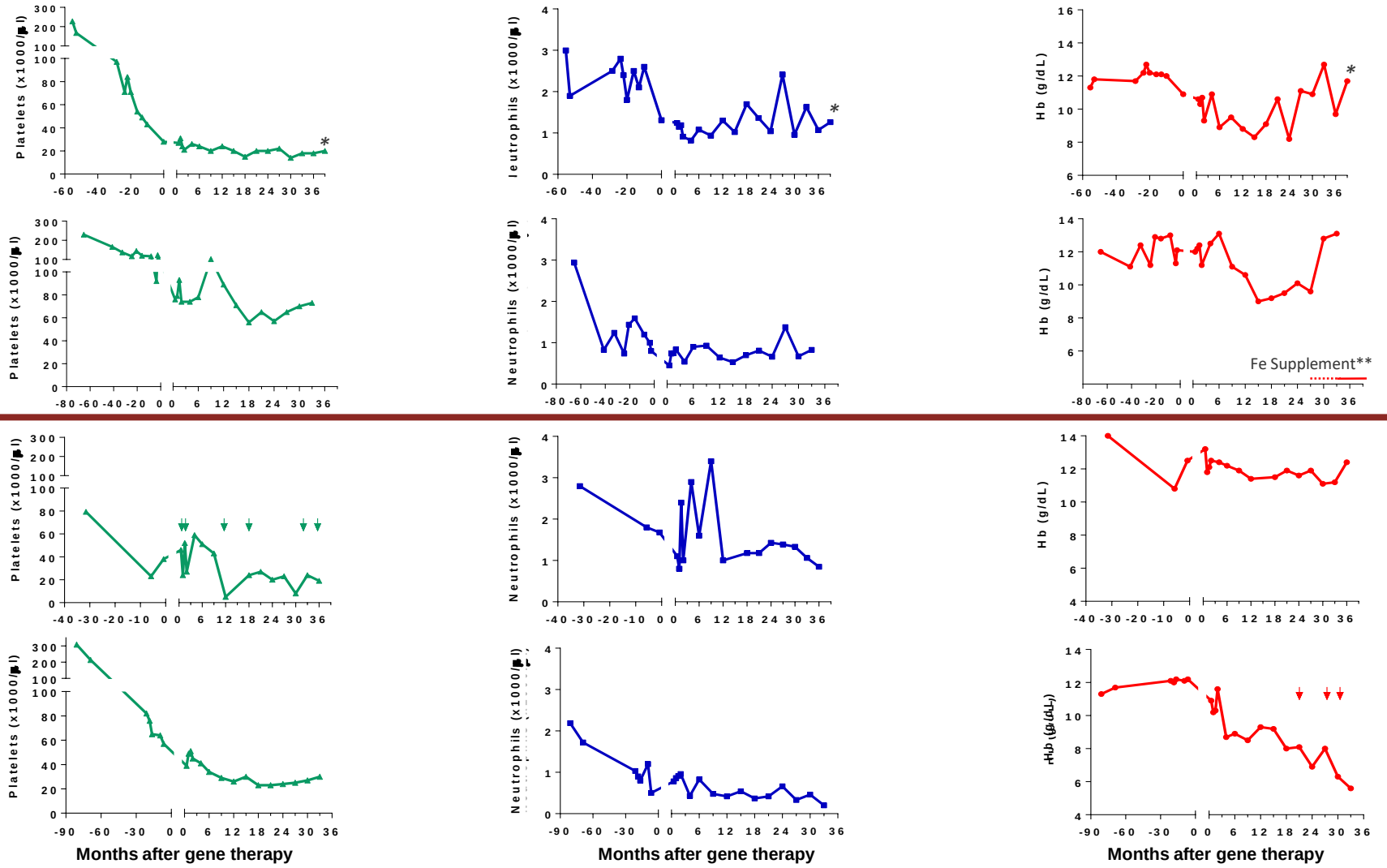
Process A: Gene Therapy Stabilizes and Improves Previously Declining Blood Counts

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

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

02005 (Fresh)
2.3x10^5 cCD34$^+$/Kg
2.8x10^3 cCFU/Kg

02004 (Cryo)
1.7x10^5 cCD34$^+$/Kg
6.9x10^3 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
** Iron supplement is not likely to enable Hb increase in the absence of viable and productive HSPCs

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