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ASH: Breakout Session

December 8, 2019

Q&A Panelists

Brian Beard, Ph.D.: AVP, Chemistry Manufacturing and Control, LVV & AAV, Rocket Pharmaceuticals

Agnieszka Czechowicz M.D., Ph.D.: Assistant Professor of Pediatrics, Division of Stem Cell Transplantation and Regenerative Medicine at the Stanford University School of Medicine (FA)

Donald (Don) Kohn, M.D.: Professor of Microbiology, Immunology and Molecular Genetics, Pediatrics Molecular and Medical Pharmacology Professor at UCLA and Principal Investigator (LAD-I)

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Jonathan Schwartz, M.D.: Chief Medical Officer & SVP, Clinical Development, Rocket Pharmaceuticals

Gaurav Shah, M.D.: Chief Executive Officer and President, Rocket Pharmaceuticals

John Wagner, M.D.: Director, Institute for Cell, Gene and Immunotherapeutics, University of Minnesota and Professor Scientific Advisor (FA)

Agenda

1. **Opening Remarks and Introductions**
   - Claudine Prowse, PhD—SVP, Corporate Development & Strategy
2. **RP-L102: Fanconi Anemia—Gaurav Shah, MD**
   - Gaurav Shah, MD—Chief Executive Officer & President
3. **RP-L201: Leukocyte Adhesion Deficiency-I**
   - Don Kohn, MD—Principal Investigator & Gaurav Shah, MD—Chief Executive Officer and President
4. **Q&A Panel**
   - Moderated by: Claudine Prowse, PhD—SVP, Corporate Development & Strategy
5. **Closing Remarks**
   - Gaurav Shah, MD—Chief Executive Officer & President

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 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 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, 2019, filed November 8, 2019. 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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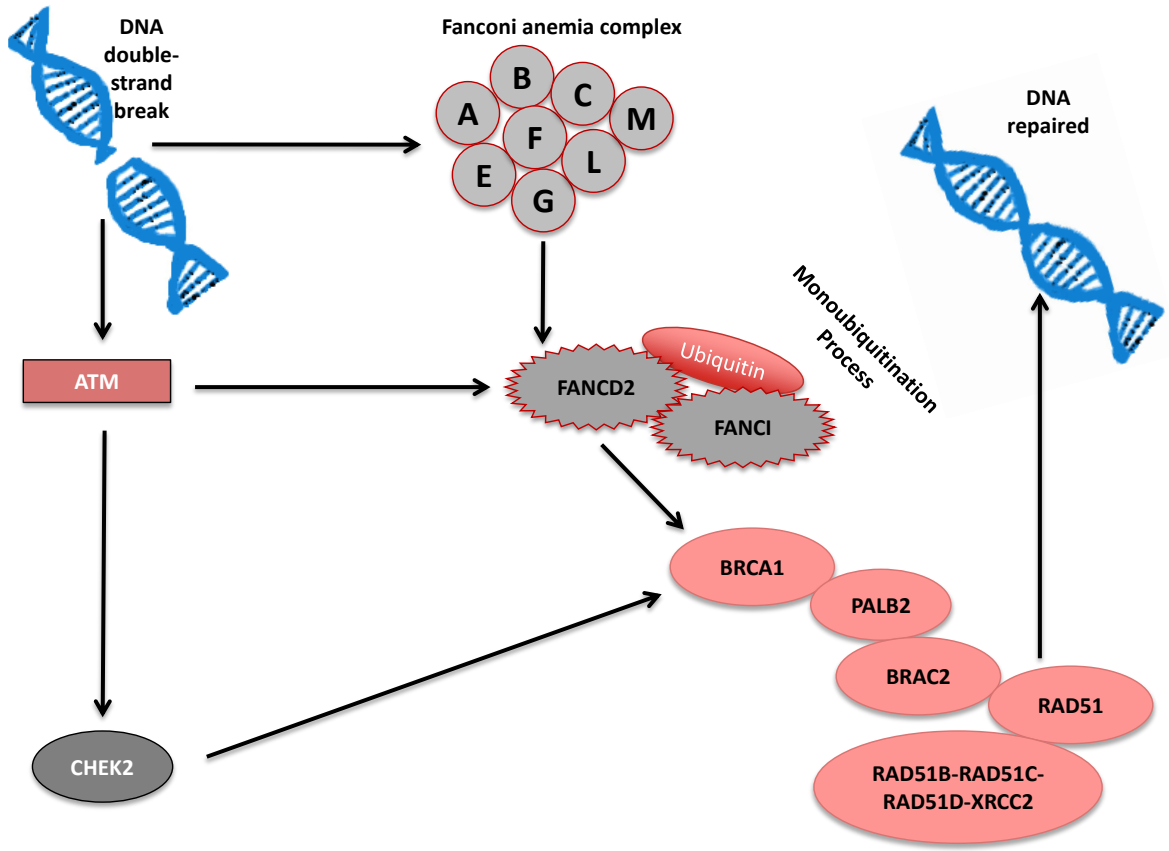
What is Fanconi Anemia (FA)?

- **Background:** Caused by mutations in FANC family of genes which are involved in DNA repair
  - FANCA mutations: 60-70% of cases
  - Disruption of FA pathway permits accumulation of interstrand cross-links; results in abnormal cell death and/or uncontrolled cell growth
- **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 hematopoietic stem cell transplant associated with 100-day mortality, GVHD, and additional increased cancer risk
- **Addressable Market[2]:** Estimated U.S.+EU target population of approximately 4,000 patients, 500 patients/year



[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 in FA

FANCOLEN-I is a Phase 1/2 clinical trial initiated by our academic partners at CIEMAT in Spain to evaluate safety and efficacy of the infusion of autologous CD34+ cells transduced with a lentiviral vector consisting of a lentiviral vector carrying the FANCA gene

- The study utilizes **Process A**
- 9 pediatric patients (ages 3-7 years) treated with Process A
- Patients had varying degrees of bone marrow health and received variations of the drug product
- Explored cryopreserved and fresh drug product

Process A Key Takeaways:[1]

- Engraftment without conditioning due to selective advantage
- Engraftment leads to clinical improvement in blood counts
- Dose-response relationship between VCN/cell dose and efficacy

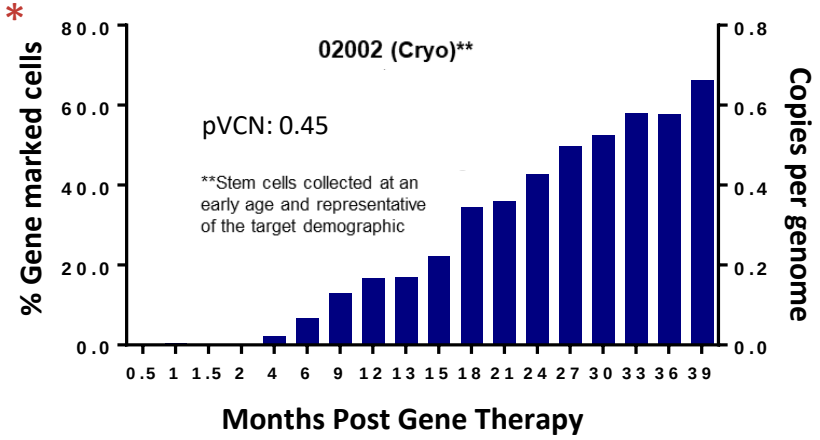
1 Four of these patients have been followed for more than 2 years (24-39 months for patients 02002, 02004, 02005, and 02006)

Process A: Bone Marrow Engraftment

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

pVCN: Product VCN

*



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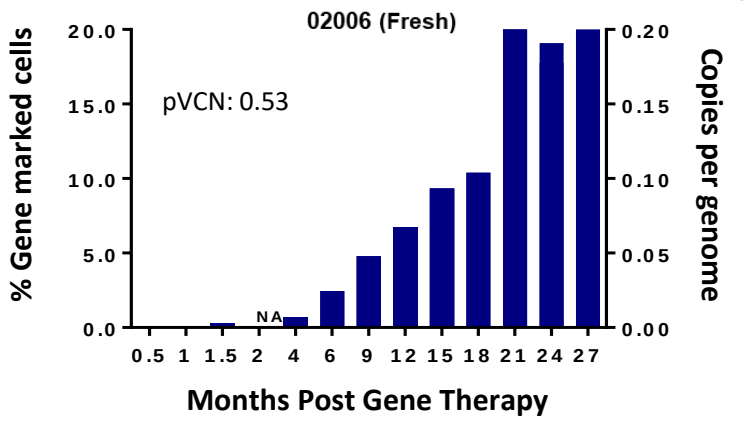
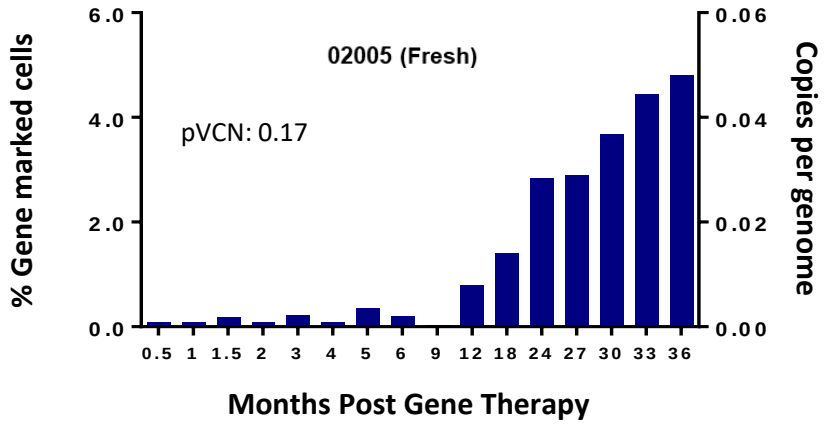
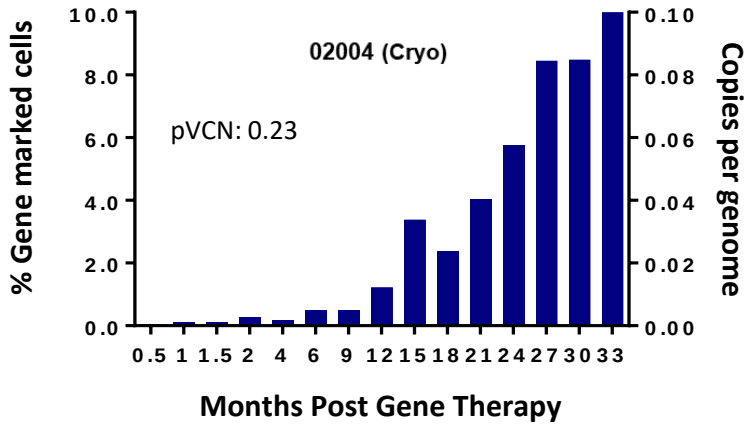




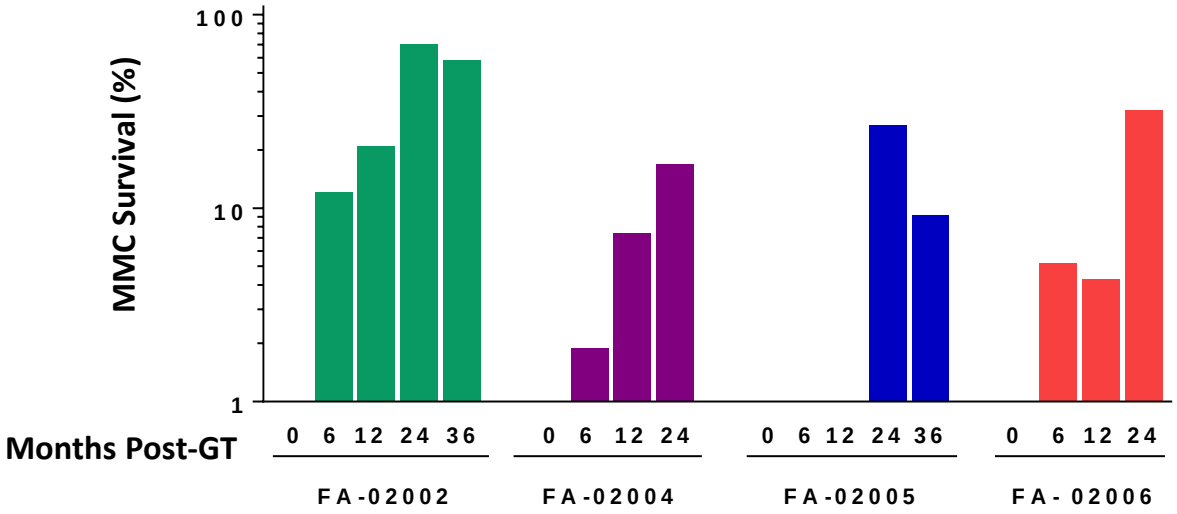


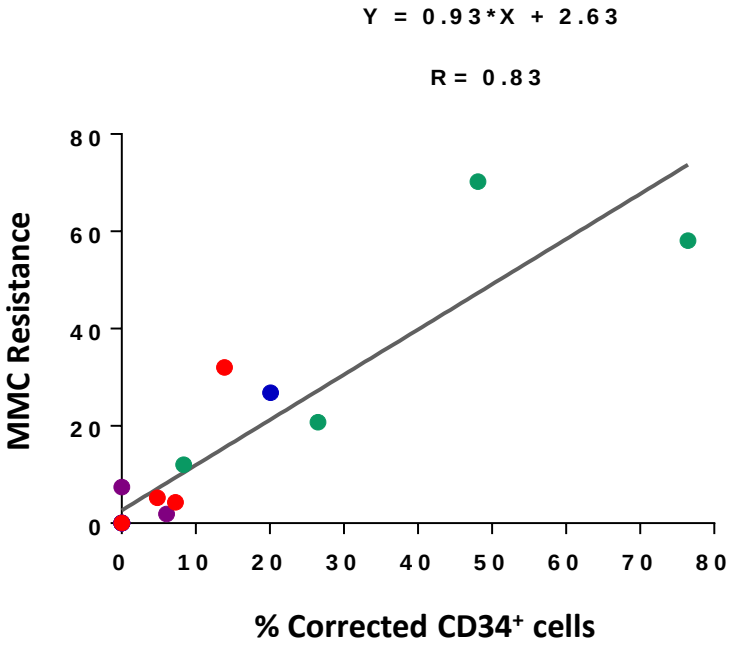
HIUNJ Data Presented at ESGCT By CIEMAT October 2019

*Minimally Acceptable Dose

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 ESGCT By CIEMAT October 2019

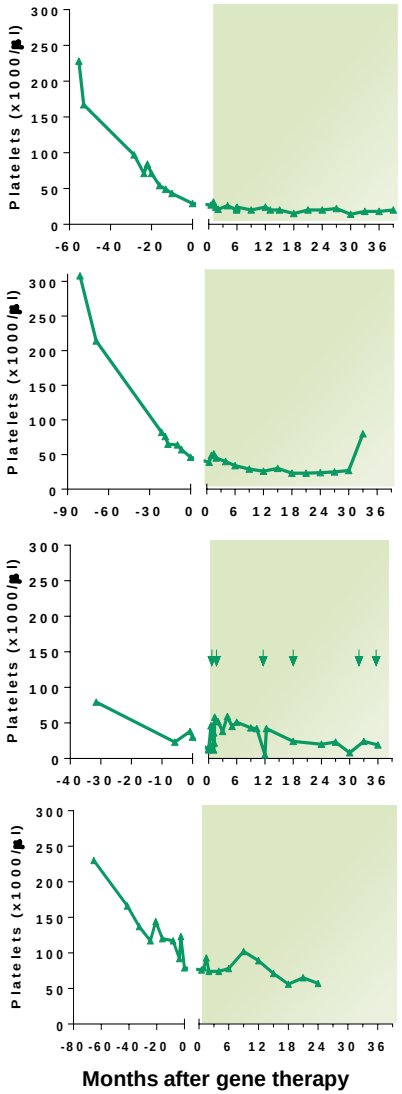
Process A: Gene Therapy Stabilizes and Improves Previously Declining Blood Counts

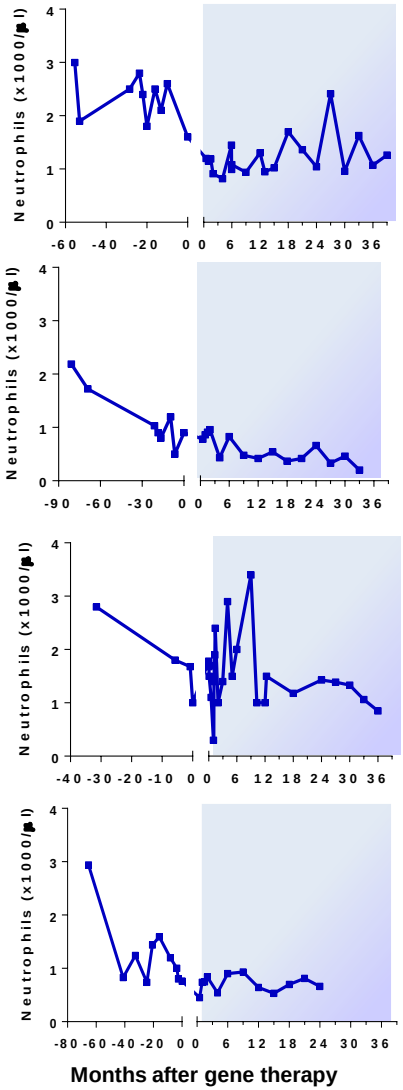
02002 (Cryo)
2.5x10^5 cCD34$^+$/Kg

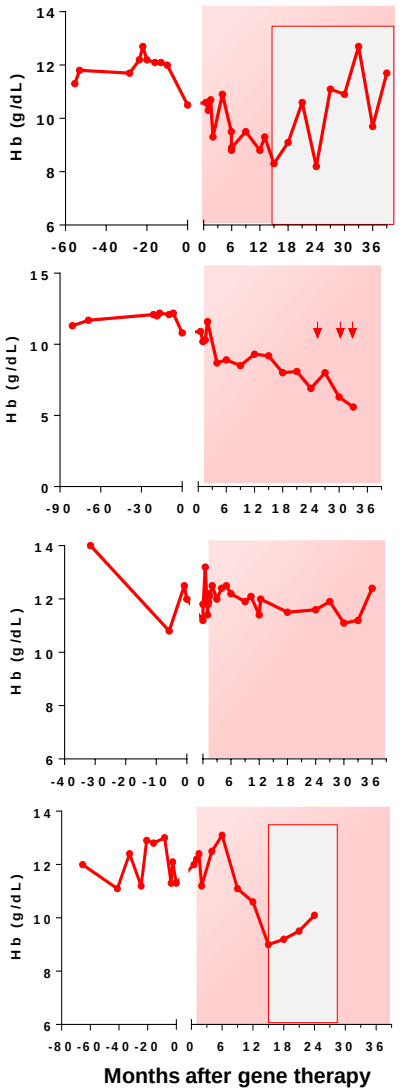
02004 (Cryo)
1.7x10^5 cCD34$^+$/Kg

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

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







HIUNJ Data Presented at ESGCT By CIEMAT October 2019

BM = Bone Marrow; cCD34+ = Corrected CD34+ cells; cCFU = Corrected Colony Forming Units

FA: Process A → Process B

CIEMAT-Sponsored FANCOLEN 1 Study

Process A

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

Optimization

Rocket-Sponsored Process B

(Cell enrichment, transduction enhancers, commercial-grade vector and modified cell processing)

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

BLA/ MAA

RP-L102 “Process B” (FA) Clinical Trial

RP-L102 Process B - U.S. Phase 1

Primary Outcomes

- To evaluate the safety of the infusion of investigational product RP-L102: autologous CD34+ enriched cells transduced with LV carrying the FANCA gene in FA-A patients

Secondary Outcomes

- Clinical response: prevention of bone marrow failure
- Engraftment as determined by peripheral blood and bone marrow vector copy number
    - Progressive increases are anticipated over time
- Phenotypic correction as evidenced by increased resistance of bone marrow and peripheral blood cells to DNA-damaging agents mitomycin-C and diepoxybutane, respectively

Inclusion Criteria

- FA complementation group A
- Age 1-12 years
- At least 1 parameter (Hb, ANC or Plt) below lower limit of normal
- Bone marrow CD34+ count ≥ 30/µL (from aspirate)
- If bone marrow CD34+ of 10-29/µL, then at least 2 of the following:
    - ▸ Hb ≥ 11g/dL ▸ PMN ≥ 900/µL ▸ platelets ≥ 60,000/µL

Exclusion Criteria

- Available & eligible HLA-identical sibling donor
- Lansky PS ≤ 60%
- MDS or leukemia (including associated cytogenetic abnormalities)
- Mosaicism with stable/improved blood counts

Lucile Packard Children’s Hospital Stanford Data Presented at ASH December 2019

RP-L102: Subject Characteristics & Drug Product Metrics

Subject Characteristics

Subject	1001	1002
Age (y) Gender	5 F	6 F
WBC (/µL)	4,000	4,600
PMN (/µL)	1,280	1,340
Hb (g/dL)	11.9	8.9
Plt (/µL)	55,000	38,000

Subject	1001	1002
MCV (fL)	86.9	106.2
BM34+ (/µL)	78	34
FA mutation	c.2606A>C, p.(Gln869Pro) c.2813A>G, p.(Asp944Gly) c.3703C>G, p.(Gln1235Glu)	c.(? -1)(522+1 523-1) del encompassing exons 1-5 c.(? -1)(283+1 284-1) del encompassing exons 1-3

Investigational Product

Subject	Nucleated Cells/kg	CD34+ Cells/kg^	CFCs/kg^	Mean VCN: Liquid Culture	Mean VCN: CFCs	CFC Survival MMC 10nM (%)
1001	7.8×10^6	2.0×10^5	5.2×10^4	2.08	1.10	33
1002	2.4×10^6	3.7×10^5	5.0×10^4	2.21	0.93*	47

* 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).

Lucile Packard Children's Hospital Stanford Data Presented at ASH December 2019

RP-L102: Favorable Benefit/Risk Profile

Adverse Events—No adverse events were associated with RP-L102 Administration

Treatment-emergent Adverse Events:

Event	Adverse Events Grade				
	any	**1**	**2**	**3**	**4**
Pyrexia	1	1	--	--	--
Croup (infectious)	1	--	--	1	--
Dyspnea	1	--	--	1	--

Drug product and patients' blood post-infusion (2d & 6m) negative for replication competent lentivirus.

Adverse events considered related to mobilization/apheresis:

- Hypocalcemia, hypoproteinemia, hypoalbuminemia, hypokalemia, nausea, fatigue, catheter site pain & bleeding and hypotension; each occurred in n=1 patient and were Grade 1
- Both patients experienced transient anemia (Hb 6.9 & 7.1 g/dL respectively) and thrombocytopenia (plt 18 & 9K/µL respectively) and received a portion of the RBC priming unit as part of apheresis procedure; 1 patient received platelet transfusion post apheresis

Lucile Packard Children's Hospital Stanford Data Presented at ASH December 2019

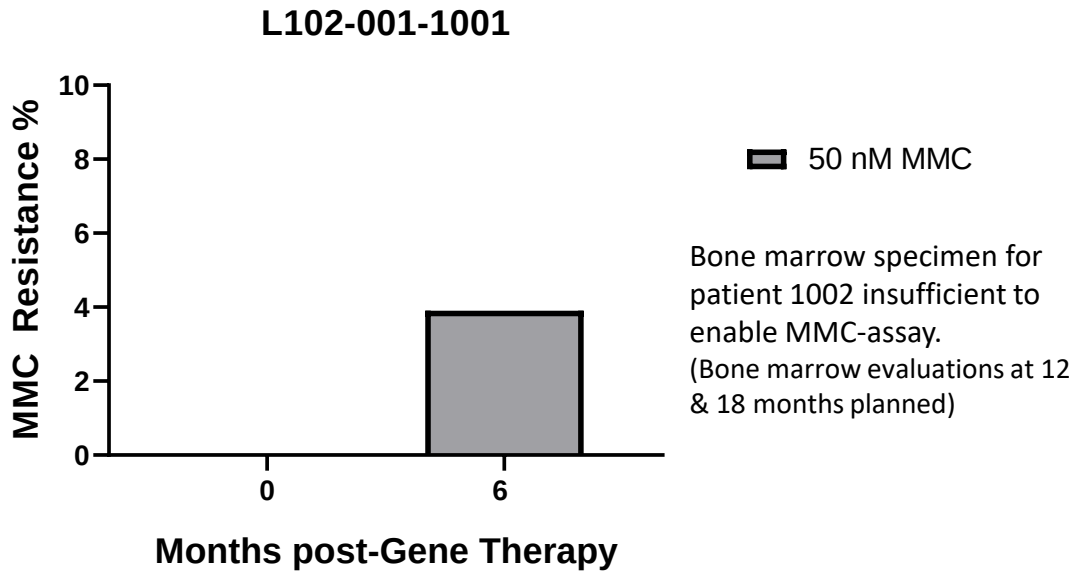
RP-L102: Preliminary Clinical Data

Vector Copy Number

Preliminary qPCR results at 4 months post infusion (PBMC):

- Pt 1001: VCN ~0.01 (1% correction)
  Pt 1002: VCN ~0.01 (1% correction)
- For patients on initial FANCOLEN-I trial who received optimal cell/CFC doses and VCNs (patients 2002 & 2006), PB VCNs at this early timepoint were similar
- In absence of conditioning, early kinetics of engraftment post-gene therapy are highly dependent on patient baseline bone marrow; increases in VCN are anticipated over ≥ 12month timeframe

Bone Marrow MMC-resistance



Resistance to 50nM MMC was demonstrated in 4% of bone marrow progenitors (CFCs) from patient 1001 at 6 months post-infusion. No resistance to this level of MMC was observed at pre-treatment baseline

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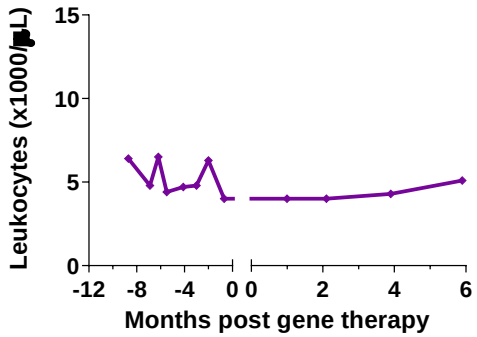
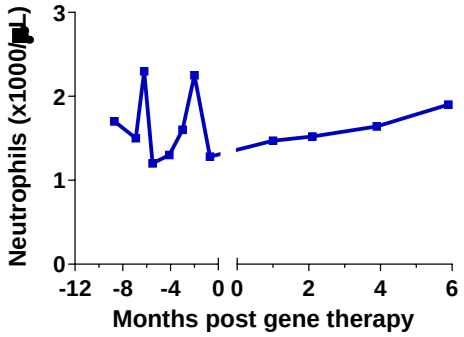
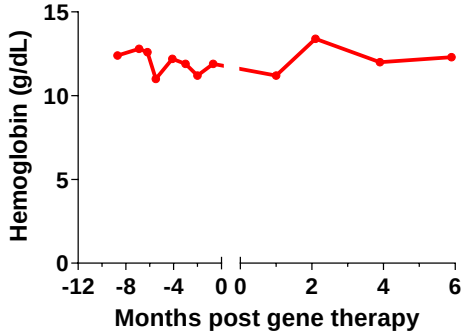
RP-L102: Preliminary Clinical Data

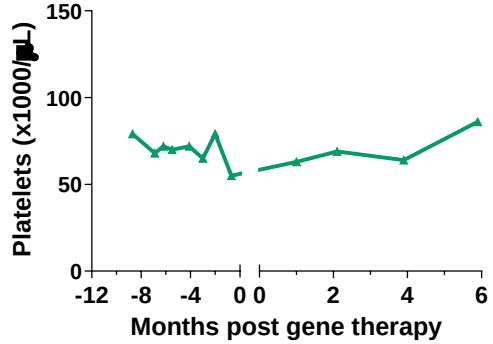
Blood Counts: Before and 6-Months After Receiving Therapy

Patient 1001

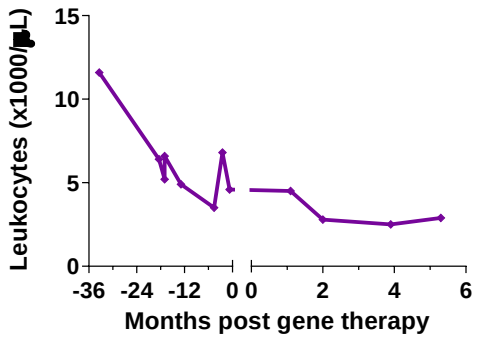


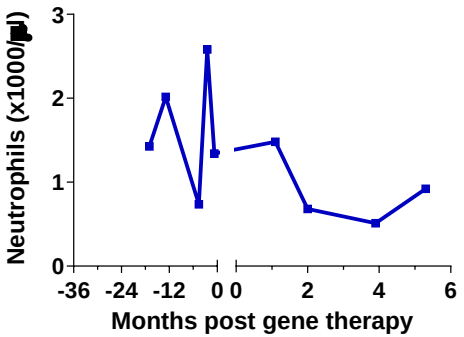


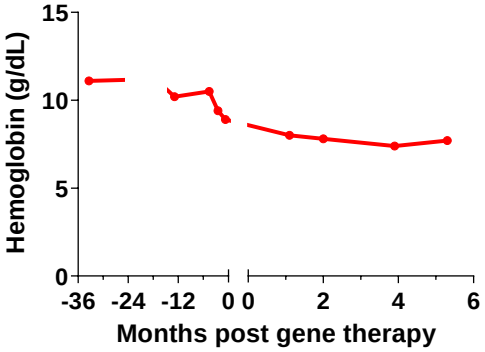


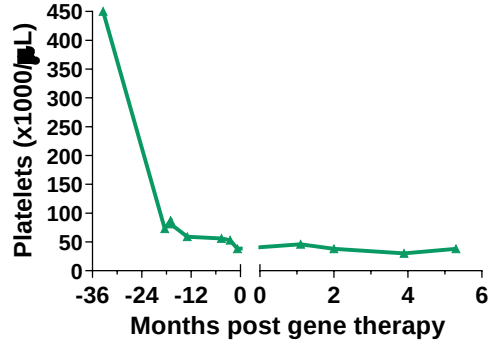


Patient 1002









- Blood count stability in both patients over 6 months following infusion, with trend increases (patient 1001 months 0⇢6; patient 1002 months 4⇢6)
- Blood count decreases in multiple lineages in both patients prior to infusion (patient 1001 over 36 months pre-Rx; patient 1002 over 9 months pre-RX)

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RP-L102: Conclusions—Drug Product

Conclusion 1.1

Investigational product metrics show consistency with parameters comparable or favorable relative to earlier processes:

- Liquid culture VCNs >2.0 and CFC VCNs ~1.0
- CFC resistance (10nM MMC) in 30-50% range
- VCNs were 2-3 fold improved while CD34+ and CFC counts were comparable to FANCOLEN-I pts who received optimal product and demonstrated engraftment, phenotypic correction and hematologic stability/improvement over 24-36 months

This US Phase 1 trial confirms the HSPC collection, transduction and viability demonstrated in the FANCOLEN-I clinical study and establishes the safety and feasibility of commercial Process B vector/cell manufacturing in FA

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RP-L102: Conclusions—Clinical

Conclusion 1.2

At 6 months, both patients are clinically stable with early indicators of engraftment in the absence of conditioning:

- Preliminary gene marking (VCN) in PB at 4 months (qPCR)
- Increasing bone marrow MMC-resistance at 6 months
- Blood counts stable (potential increase) at 6 months, in setting of multi-lineage decreases in 9-36 months prior to gene therapy

With a demonstrated favorable safety profile and early indication of efficacy, global Phase 2 study is underway: NCT# NCT04069533

- ***Initial patient received infusion***
- ***Registration-enabling study with primary endpoint of bone marrow MMC-resistance at 1-3 years post-infusion***

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RP-L201: Leukocyte Adhesion Deficiency-I

LAD is CIRM funded

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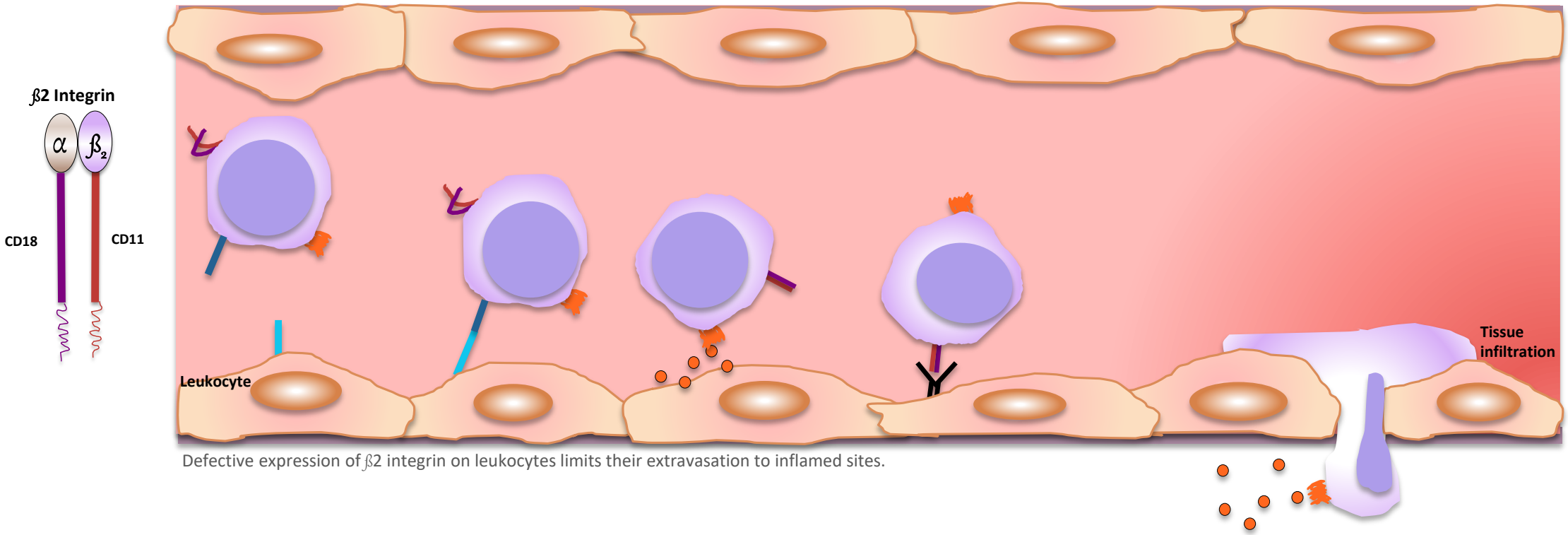
What is Leukocyte Adhesion Deficiency-I (LAD-I)?



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

- **Background:** In LAD-I, mutations in the common chain (CD18) of the beta2-integrin family prevent dimerization and expression of heterodimers on cell surface essential for cell migration and adhesion.
  - Each of the beta2-integrins is a heterodimer composed of an alpha chain (CD11a, CD11b, or CD11c) noncovalently bound to CD18 (common beta2-subunit).
  - Thus, defects in CD18 expression result in very low or no surface expression of CD11
- 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 GVHD

Clinical Pathogenesis of LAD-I

LAD-1 Disease Spectrum

Moderate:
2-30%
CD18+ PMN

Severe:
<2%
CD18+ PMN

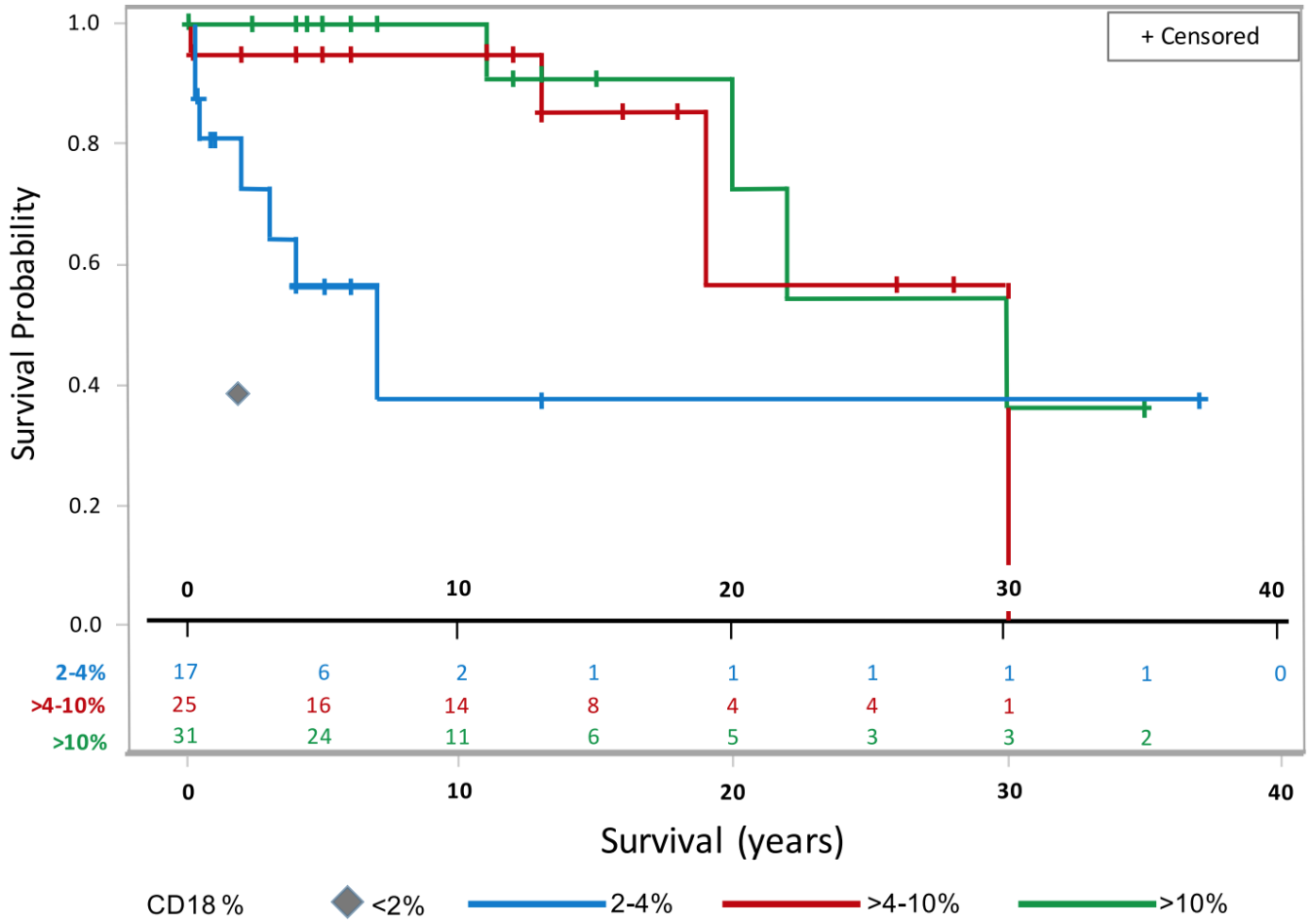
PMN = polymorphonuclear neutrophils

LAD-1 Clinical Prognosis

- Patients suffer from recurrent infections; fatal in majority
  - 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[1]

-Patients with moderate 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 (LAD-I): Clinical Trial and Outcome Measures[1]

Trial Design – Non-Randomized Phase 1/2 Study

Enroll 9 patients globally across Phase 1/2

Phase 1: Enroll two patients to assess safety and tolerability (n=2)

Phase 2: Overall survival at multiple sites (U.S. and EU) (n=7)

Primary Outcomes

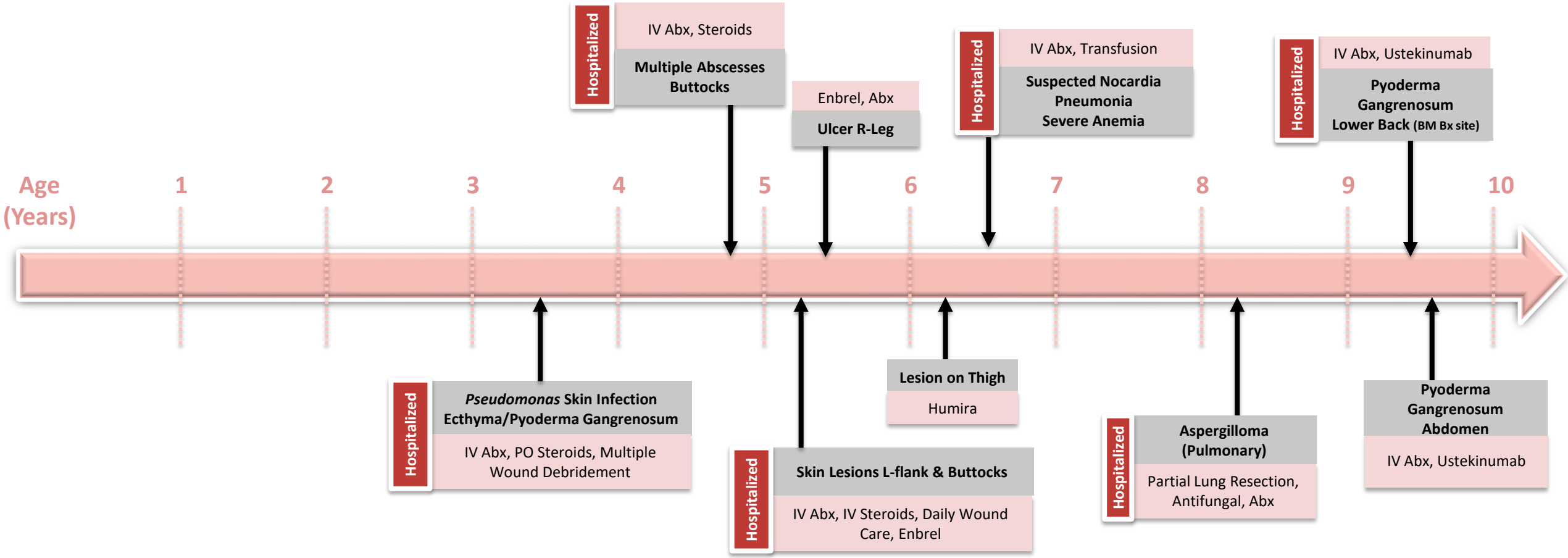
- Phase 1:
  - Safety associated with treatment
- Phase 2:
  - Survival: proportion of patients alive at age 2 and at least 1-year post infusion
  - Safety associated with treatment

Secondary Outcomes

- Percentage of neutrophils expressing at least 10% CD18
- At least 10% of peripheral blood neutrophils carrying the therapeutic lentiviral vector at 6 months post-infusion
- Incidence and severity of infections
- Improvement/normalization of neutrophils
- 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

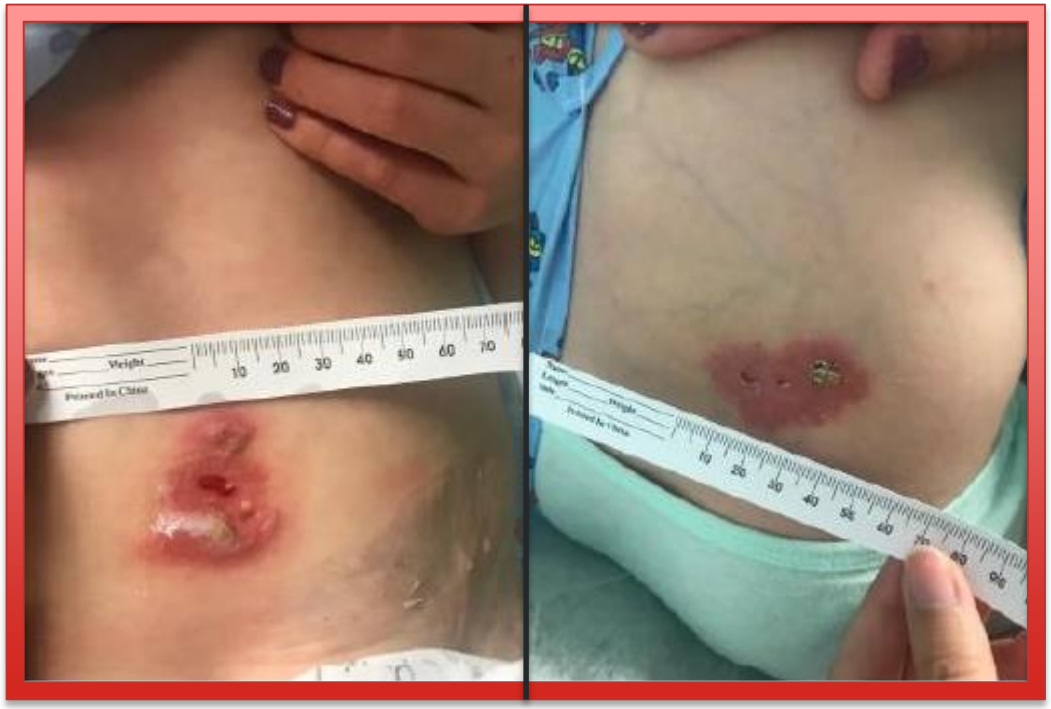
Medical History of Patient L-201-003-1001



Historical patient records collected by UCLA Mattel Children's Hospital

RP-L201: Visible Improvements Post-Treatment

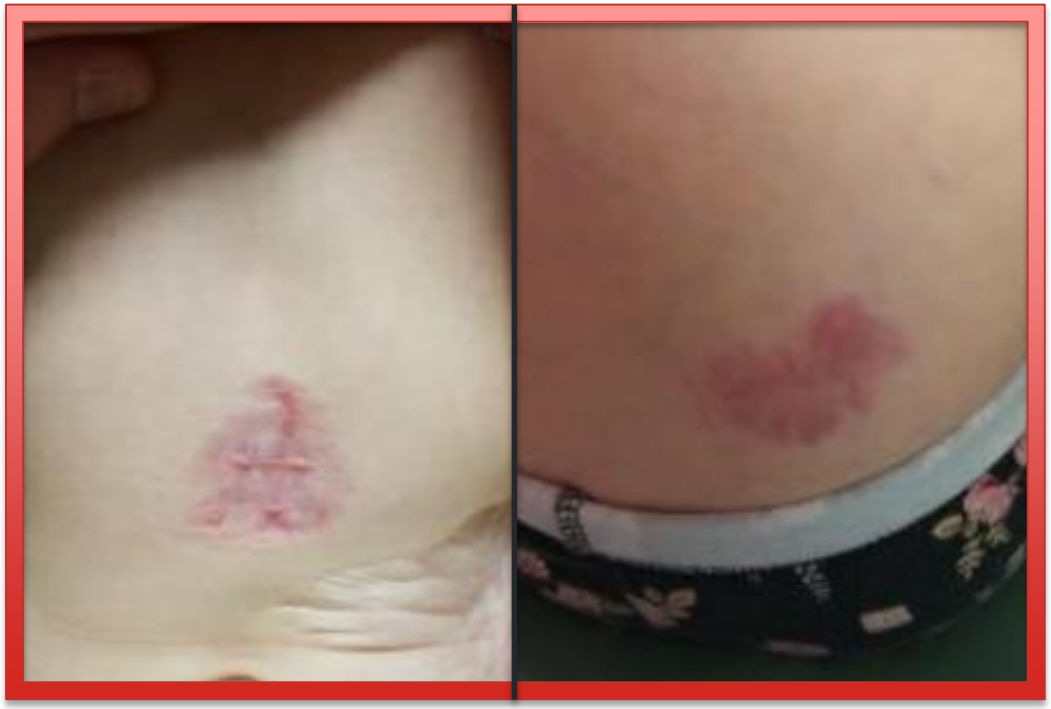
Prior to Gene Therapy—At Baseline



Spontaneous Abdominal Lesion

Lower Back Lesion (after BM aspirate)

Post Gene Therapy—At 3-Months



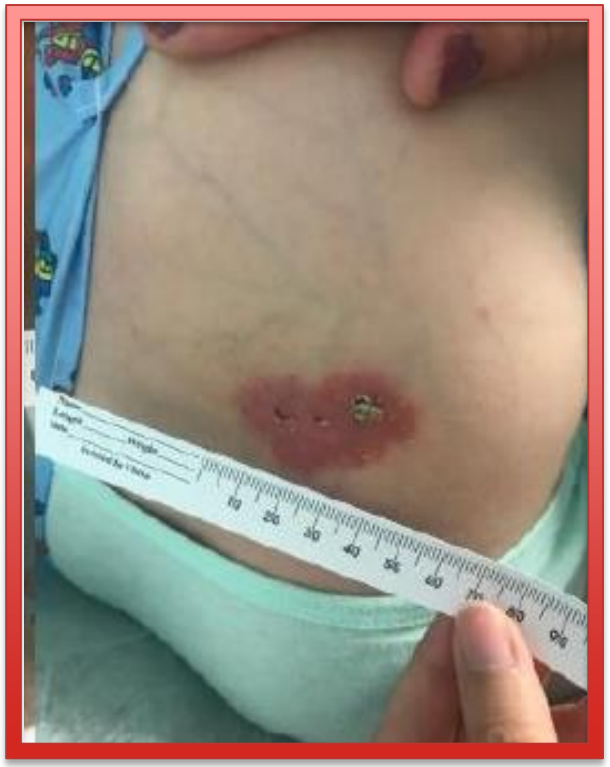
Spontaneous Abdominal Lesion

Lower Back Lesion (after BM aspirate)

UCLA Mattel Children's Hospital Data December 2019

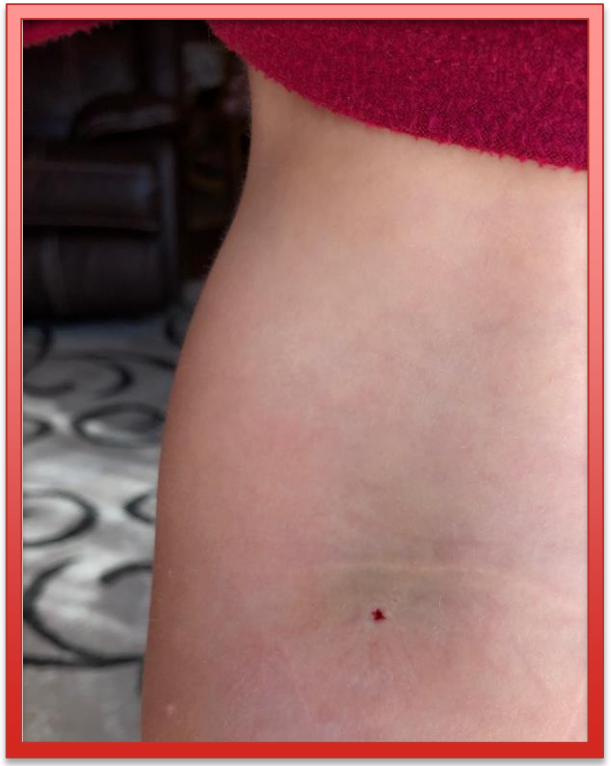
RP-L201: Visible Improvements Post-Treatment
No Infection/Inflammation After 3-Month Bone Marrow Biopsy

Prior to Gene Therapy: BM Bx Site



Lower Back Lesion
(after BM aspirate)

BM Bx Site 2 Days After 3-Month Marrow Bx



Lower Back
(after BM aspirate)

UCLA Mattel Children's Hospital Data December 2019

RP-L201: Drug Product Metric and Clinical Results

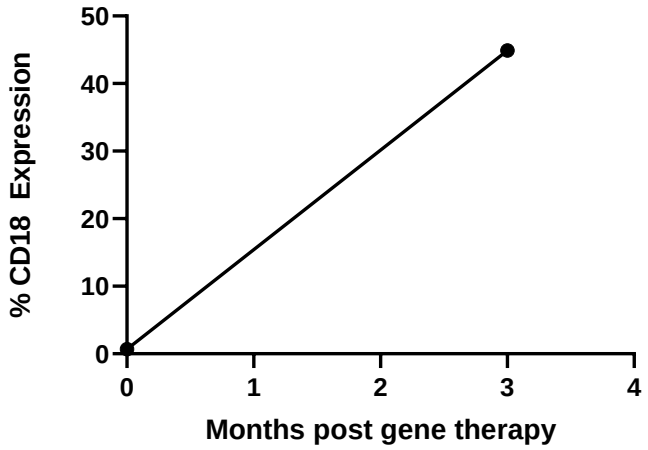
Key Drug Product Metrics

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

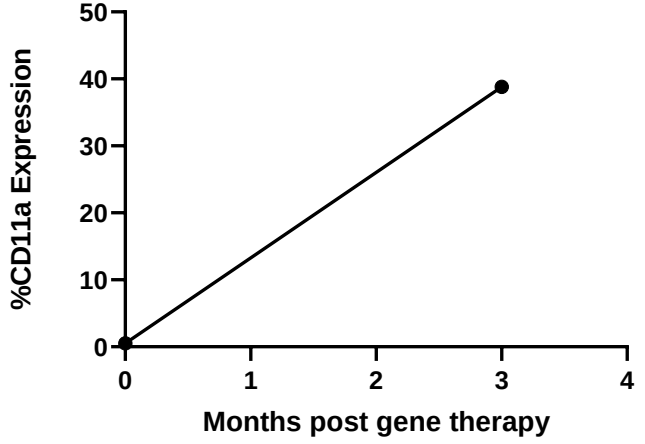
Clinical Results

- **VCN (myeloid) 3-months post-treatment**: 1.5
- **CD18 Expression in Peripheral Blood**:
  - **3-month CD18 expression post-treatment: 45%**
  - Pre-treatment CD18 expression was <1%

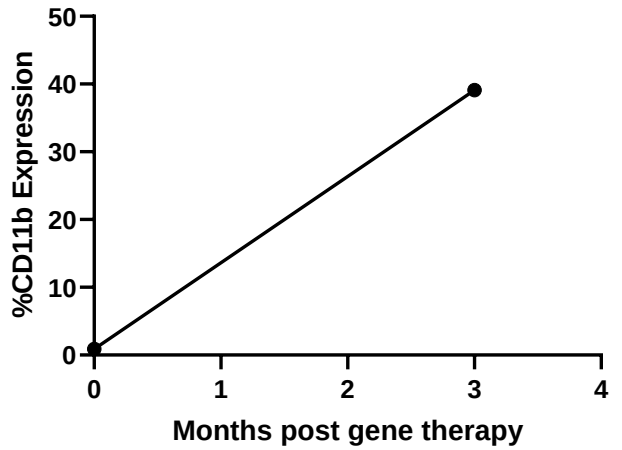
%CD18 Expression in Peripheral Blood



%CD11a Expression in Peripheral Blood



%CD11b Expression in Peripheral Blood



UCL Gosh Data December 2019

LAD-I: New Patients Identified to Date

Age	Gender	Geographic Location	Status
4 years	F	US	Enrolled
2 years	F	US	Meets eligibility criteria; awaiting enrollment
2 years	F	India	Meets eligibility criteria; obtained visa to enter US; awaiting enrollment
2 months	M	US	Flow cytometry consistent with severe LAD-I; patient will be eligible when 3 months of age
5 months	M	US	Suspected severe LAD-I; flow cytometry pending

Additional 4 patients identified who meet eligibility criteria

THANK YOU!

Q&A Panelists

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Donald (Don) Kohn, M.D.: Professor of Microbiology, Immunology and Molecular Genetics, Pediatrics Molecular and Medical Pharmacology Professor at UCLA and Principal Investigator (LAD-I)

Kinnari Patel, PharmD, MBA: Chief Operating Officer & EVP, Development, Rocket Pharmaceuticals

Paula RÍo, Ph.D.: Senior Scientist, CIEMAT, CIBERER, IIS Fundación Jiménez Díaz (FA)

Jonathan Schwartz, M.D.: Chief Medical Officer & SVP, Clinical Development, Rocket Pharmaceuticals

Gaurav Shah, M.D.: Chief Executive Officer and President, Rocket Pharmaceuticals

John Wagner, M.D.: Director, Institute for Cell, Gene and Immunotherapeutics, University of Minnesota and Professor Scientific Advisor (FA)