Rocket Program Updates for Fanconi Anemia, Pyruvate Kinase Deficiency and Leukocyte Adhesion Deficiency-I

December 14, 2021

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Various statements in this release concerning Rocket's future expectations, plans and prospects, including without limitation, Rocket's expectations regarding its guidance for 2021 in light of COVID-19, the safety, effectiveness and timing of product candidates that Rocket may develop, to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD), Infantile Malignant Osteopetrosis (IMO) and Danon Disease, and the safety, effectiveness and timing of related pre-clinical studies and clinical trials, may constitute forward-looking statements for the purposes of the safe harbor provisions under the Private Securities Litigation Reform Act of 1995 and other federal securities laws and are subject to substantial risks, uncertainties and assumptions. You should not place reliance on these forward-looking statements, which often include words such as "believe," "expect," "anticipate," "intend," "plan," "will give," "estimate," "seek," "will," "may," "suggest" or similar terms, variations of such terms or the negative of those terms. Although Rocket believes that the expectations reflected in the forward-looking statements are reasonable, Rocket cannot guarantee such outcomes. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including, without limitation, Rocket's ability to monitor the impact of COVID-19 on its business operations and take steps to ensure the safety of patients, families and employees, the interest from patients and families for participation in each of Rocket’s ongoing trials, our expectations regarding when clinical trial sites will resume normal business operations, our expectations regarding the delays and impact of COVID-19 on clinical sites, patient enrollment, trial timelines and data readouts, our expectations regarding our drug supply for our ongoing and anticipated trials, actions of regulatory agencies, which may affect the initiation, timing and progress of pre-clinical studies and clinical trials of its product candidates, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, and unexpected expenditures, as well as those risks more fully discussed in the section entitled "Risk Factors" in Rocket’s Annual Report on Form 10-K for the year ended December 31, 2020, filed March 1, 2021 with the SEC. Accordingly, you should not place undue reliance on these forward-looking statements. All such statements speak only as of the date made, and Rocket undertakes no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

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Summary of Rocket Lentiviral Program Updates and Status

Fanconi anemia (Pivotal Phase II):

- 6 of 8 initial patients with ≥12 months follow-up display evidence of engraftment
  - Increasing BM MMC-resistance and BM/PB VCN
  - BM MMC-resistance appears associated with hematologic stability
- No conditioning, favorable safety with single RP-L102 related (grade 2) SAE

Pyruvate Kinase Deficiency:

- One-year follow-up in both initial adult patients indicates ongoing normal-range Hb (pre-treatment levels 7-7.4g/dL)
- Bilirubin, erythropoietin and other hemolysis markers also improved substantially
- Anemia resolution and transfusion-independence are accompanied by improved QOL improvements

Leukocyte Adhesion Deficiency-I (Pivotal Phase I/II):

- Accrual complete: n=9
- Engraftment in all 9 patients and phenotypic reversal in 8 of 8 patients with ≥3m follow-up
- Restored CD18 expression accompanied by CD11 expression, VCN, normalized WBC, markedly reduced infections
- Favorable safety, no RP-L201 related SAEs; no graft failure or GVHD

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Gene Therapy for Fanconi Anemia (Group A): Preliminary Results of Ongoing RP-L102 Clinical Trials

Jonathan Schwartz, M.D.
Chief Medical Officer, Senior Vice President

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Current Status of Patients

11 patients have received RP-L102

8 patients with 12-32 months of follow up

3 patients with < 12 months of follow up*

Included in efficacy parameter analyses:

n=7

- **VCN**
- **BM CFC MMC-res**
- **Blood Counts**

1 (1001)
3 (2004)
4 (2008)
5 (2009)
6 (2010)
7 (2011)
8 (2014)
2 (1002)‡

n=1

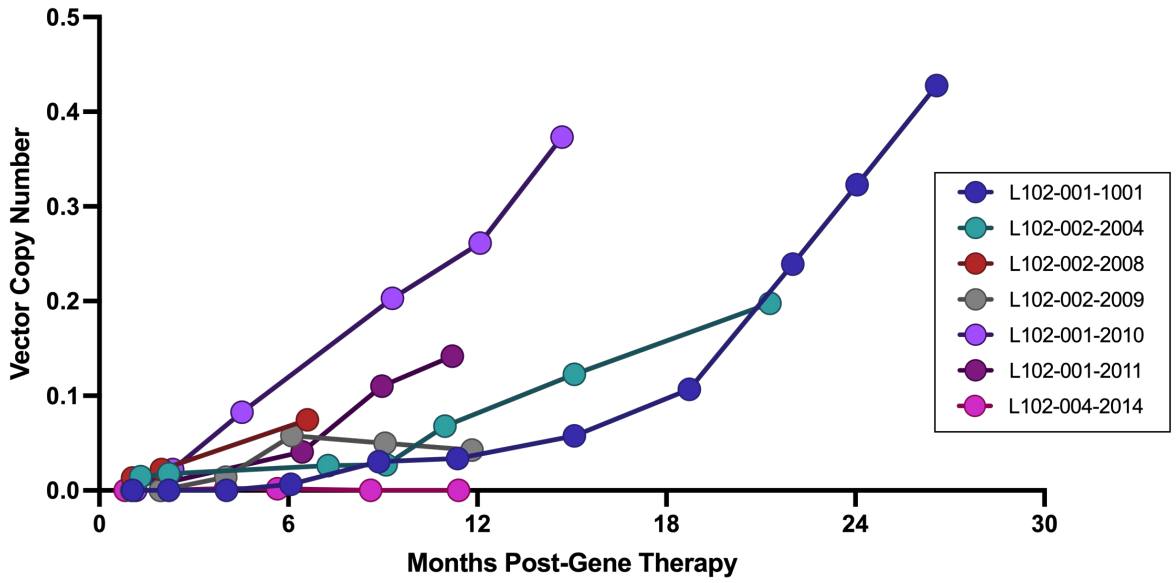
9 (2016)
10 (2021)
11 (2023)

n=11

***Reflects one additional patient treated after ASH data cutoff 1-NOV-2021**

‡ Patient 2 (1002) was withdrawn from the study at 18m due to bone marrow failure (BMF) requiring alloHSCT.

RP-L102 Study Patients with ≥12m Follow Up Demonstrate Evidence of Genetic and Phenotypic Correction



- **Sustained PB VCN in 6 of 7 currently enrolled patients with ≥ 12 months of follow up**
- **Concomitant BM CFC MMC resistance ≥ 10% above baseline values**

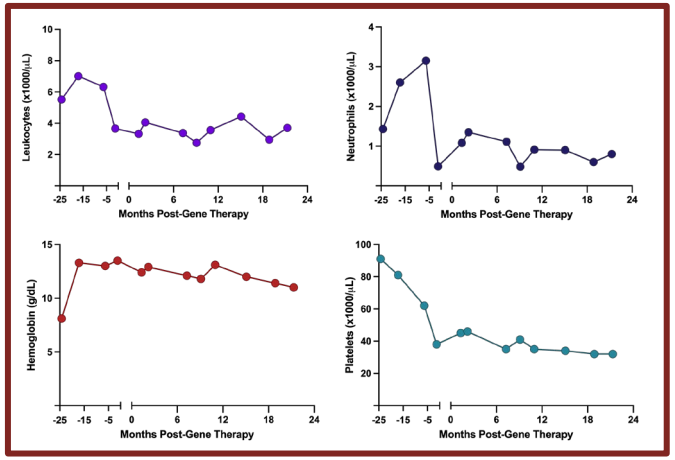
NOTE: Efficacy (defined as MMC resistance ≥ 10% at two or more timepoints) in 5 of 12 patients required to reject null hypothesis.

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

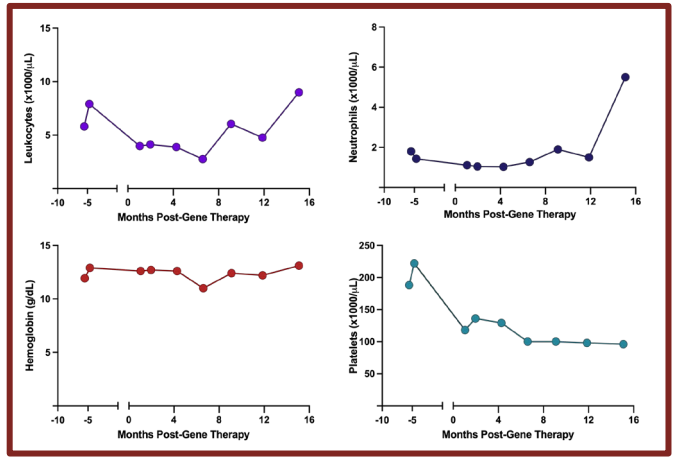
† Assessment was not performed at study's centralized laboratories

Blood Count Stabilization in at Least 5 Patients with BM CFC MMC Resistance ≥10%

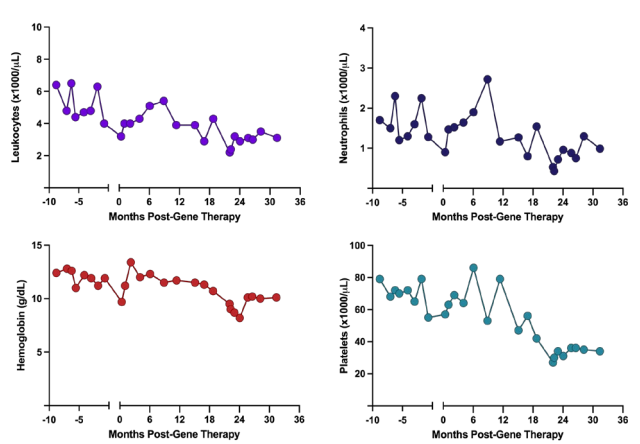
Subject 3 (2004)



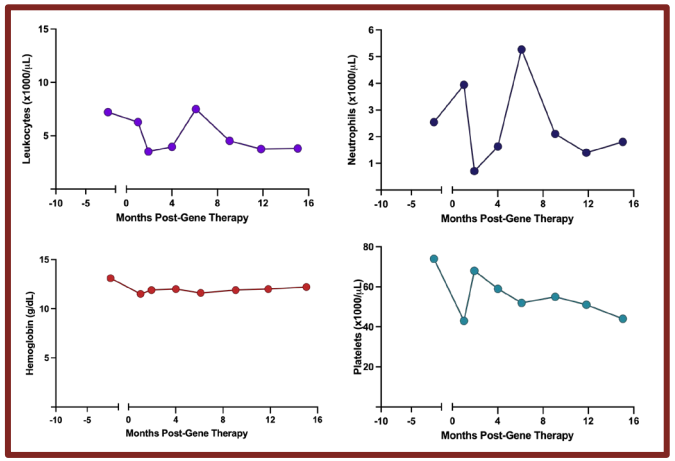
Subject 4 (2008)



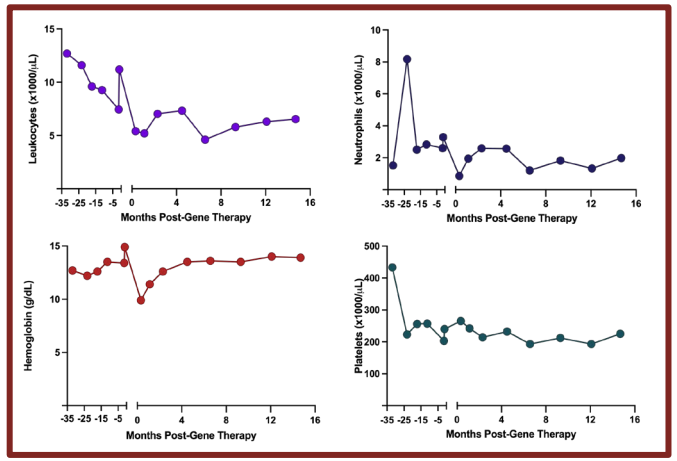
Subject 1 (1001)



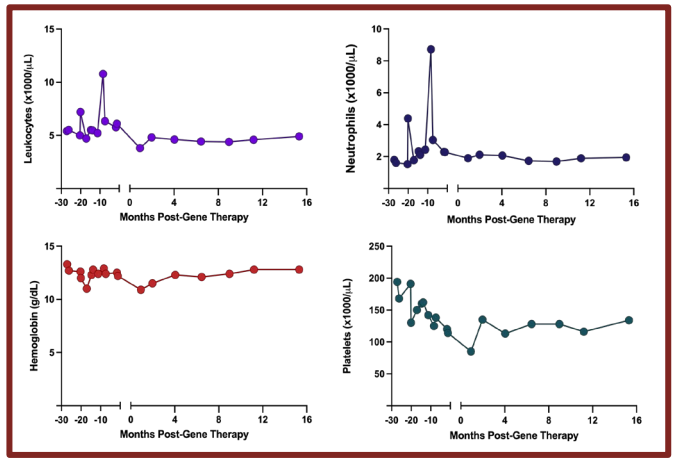
Subject 5 (2009)



Subject 6 (2010)



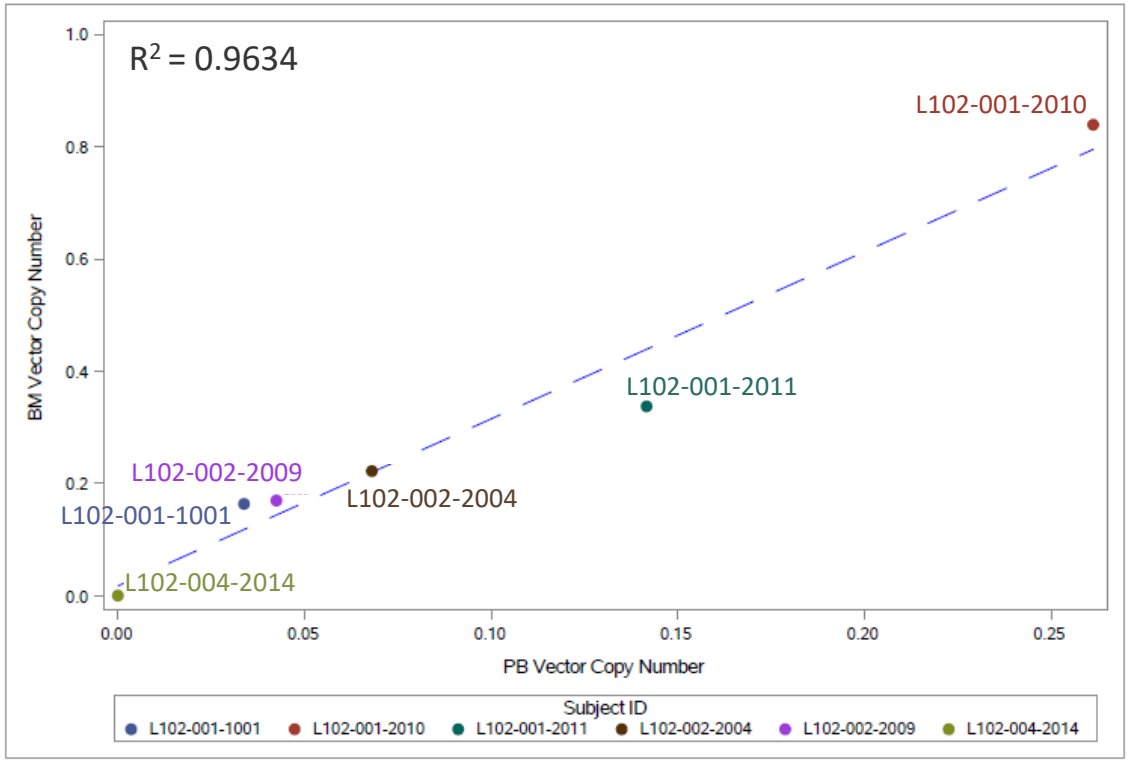
Subject 7 (2011)



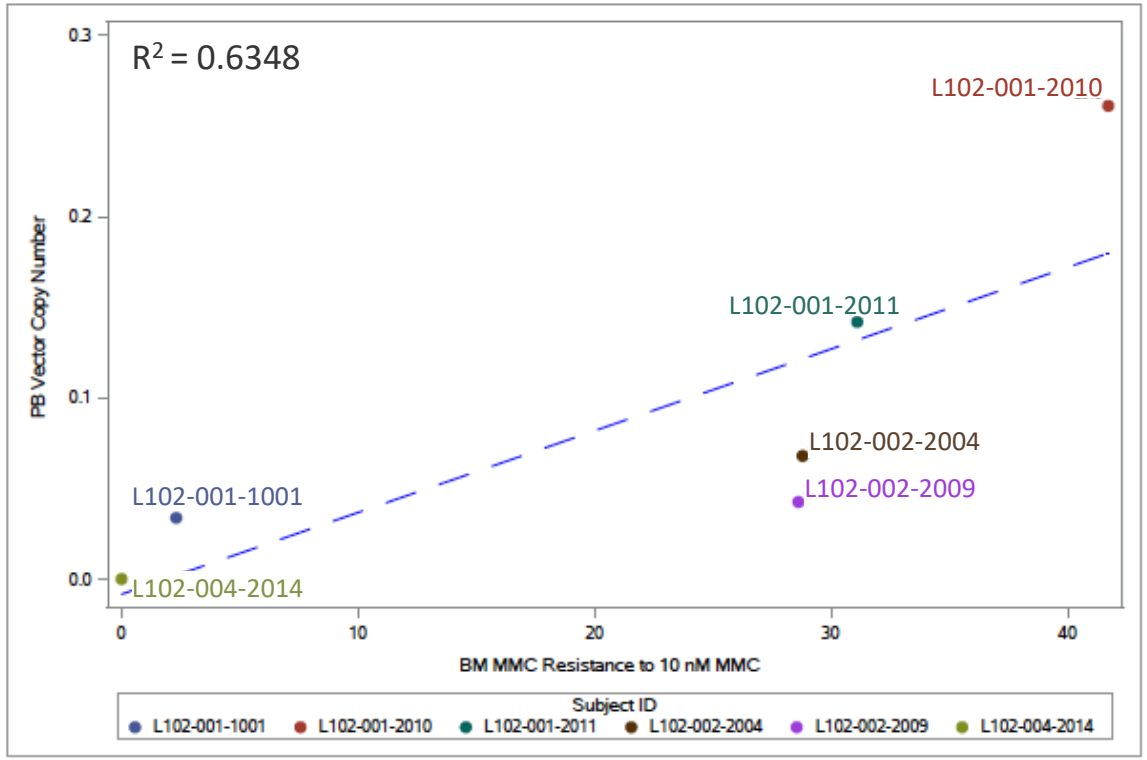
- **Blood count stabilization in 5/5 patients with BM CFC MMC Resistance ≥ 20%**
- **Patient 1 (1001; MMC-res 16%) had modest decline in blood counts with potential stabilization after ~18m; no transfusions have been required**

Preliminary Associations Between BM & VCN Efficacy Parameters at 12m

PB VCN vs. BM VCN at 12 months



BM MMC-Resistance vs. PB VCN at 12 months



Fanconi Anemia Summary

- Of 11 patients treated to date:
  - **6 of 8 patients with ≥12m of follow-up display evidence of engraftment**
    - 1 patient's course (1002) complicated by *Influenza B* infection & BMF; required BMT and engrafted without complications
- Increasing BM CFC MMC resistance seen in 6 patients*
- Initial analyses suggest that post-treatment bone marrow MMC-resistance and VCN are associated with peripheral blood genetic correction
- Safety profile of RP-L102 appears favorable
  - Patients treated without conditioning
  - No signs of dysplasia or other concerning features
  - RP-L102 related SAEs: 1 infusion-related reaction (transient, Grade 2)

MMC resistance ≥ 10% at <u>two</u> or more timepoints in 5 of 12 patients required to reject null hypothesis.

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Pyruvate Kinase Deficiency: Updated Results of a Global Phase 1 Study for Adult and Pediatric Patients

Kinnari Patel, Pharm.D., M.B.A.
Chief Operating Officer, President

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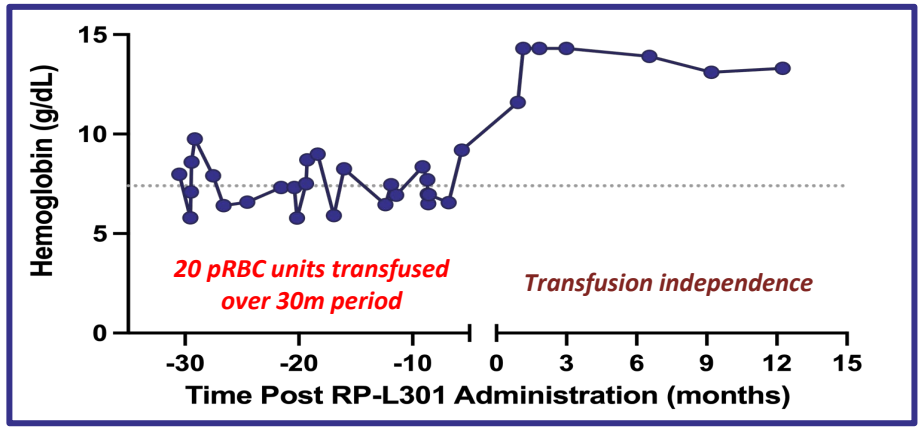
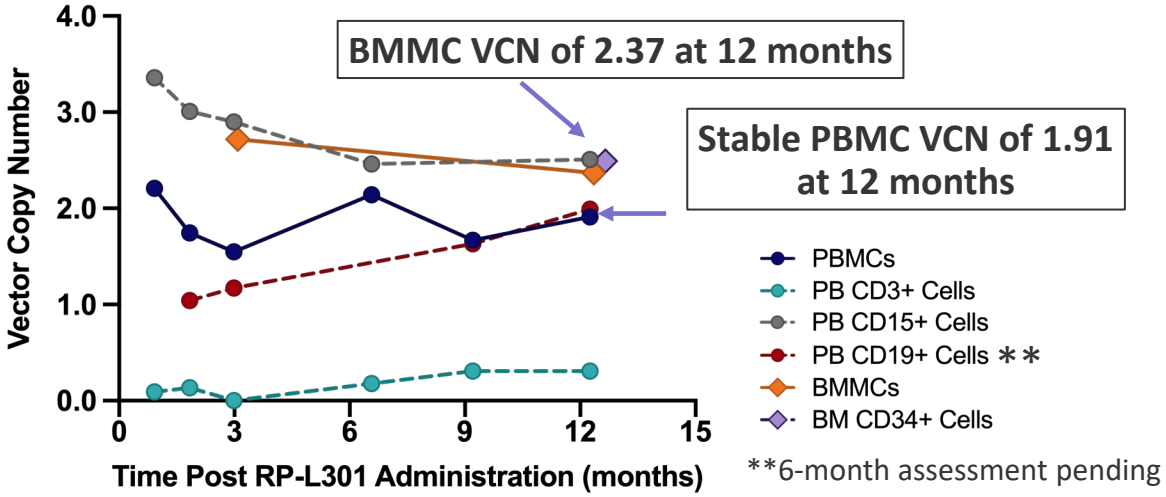
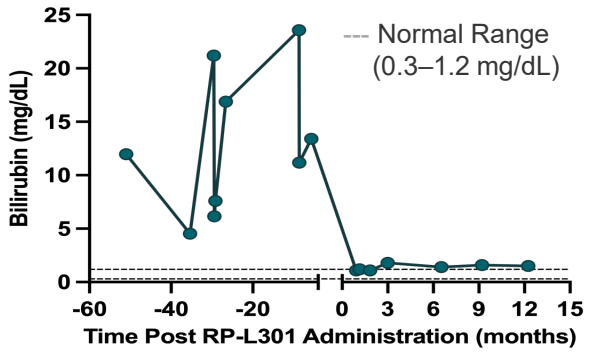
Preliminary Efficacy Results: L301-006-1001



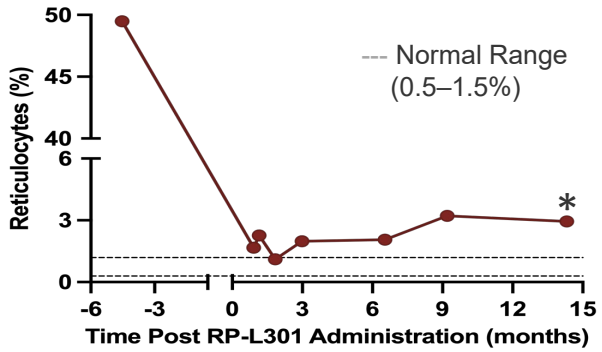
- Sustained **hemoglobin improvement from ~7.4 g/dL to 13.3 g/dL** over 12 months post-RP-L301 infusion
- No red blood cell transfusion requirements following engraftment



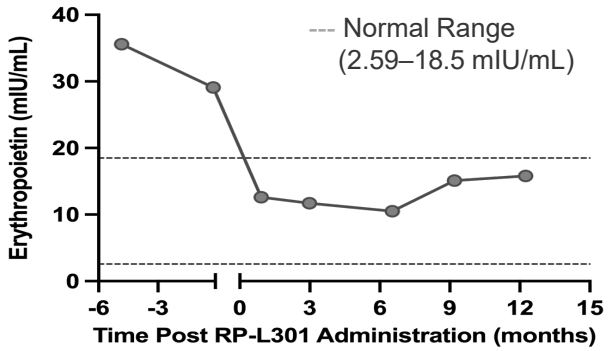
**6-month assessment pending



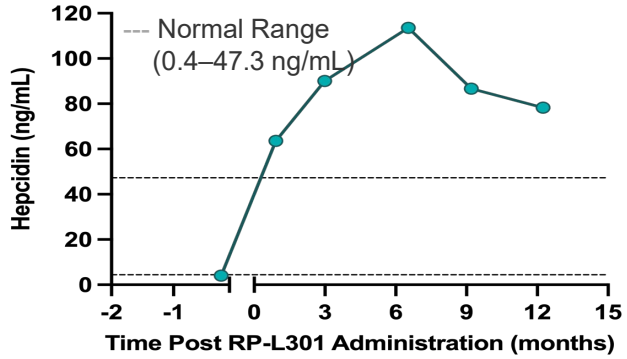
Significant bilirubin decrease from 13.4 mg/dL to 1.5 mg/dL



Sustained reduction in reticulocytes



Continued erythropoietin normalization



Hepcidin increased from <4.0 ng/mL to 78.3 ng/mL

* Reticulocytes not obtained at 12-month visit; obtained locally at ~14 months post-infusion

NOTE: Data cut-off as of November 3, 2021

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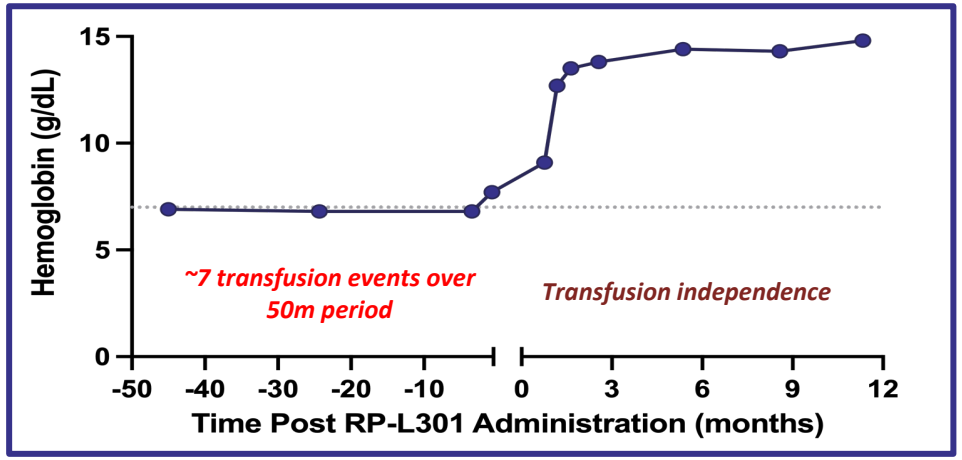
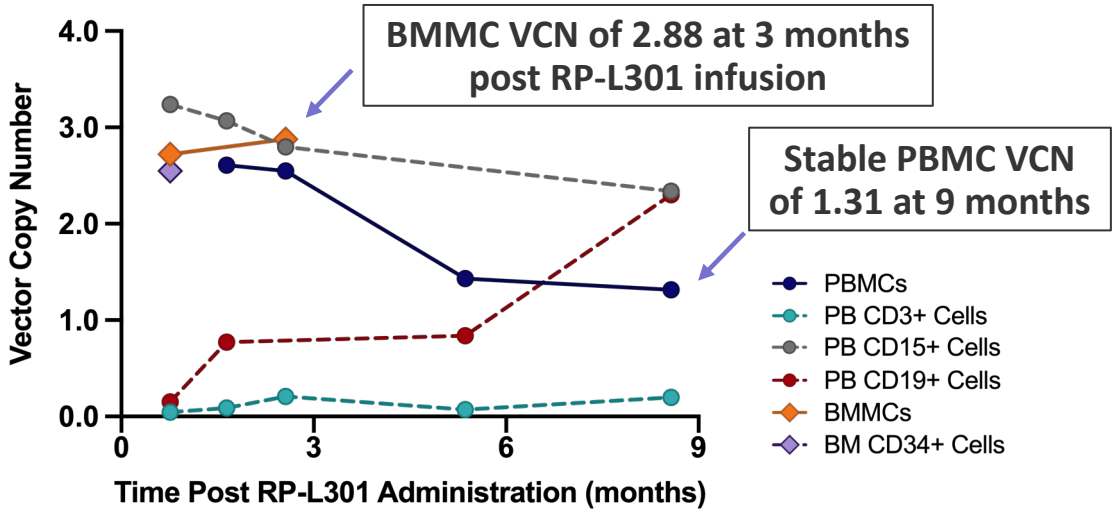
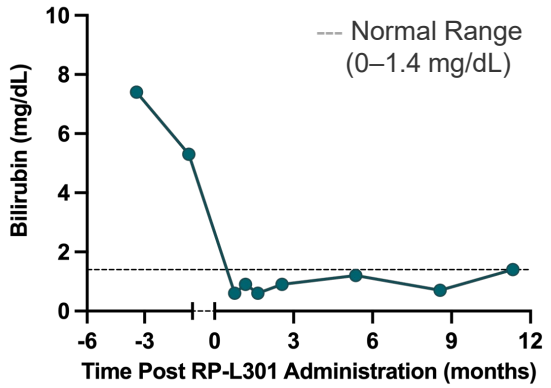
Preliminary Efficacy Results: L301-001-1002

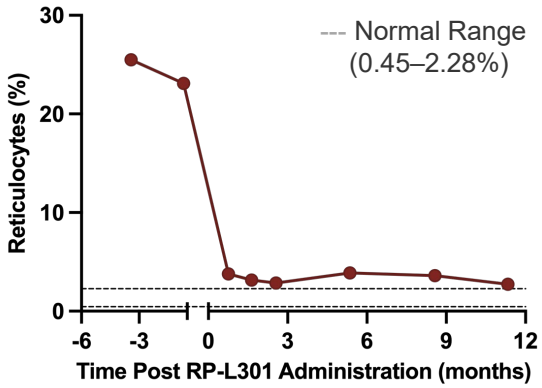


- **Hemoglobin normalized to 14.8 g/dL** at ~12 months post-rx
- No red blood cell transfusion requirements following engraftment

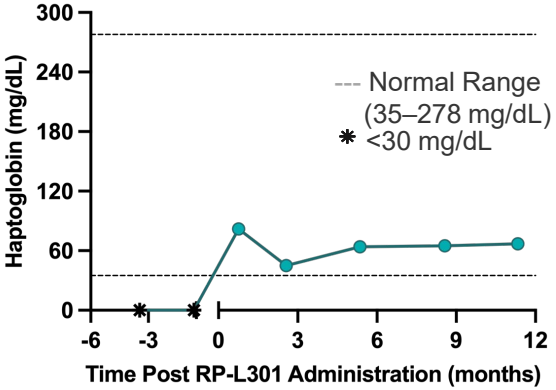




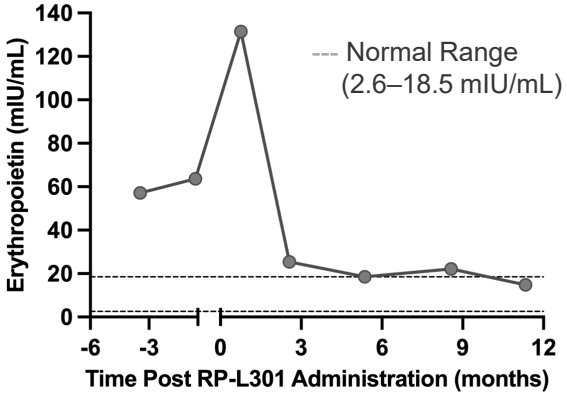
Normalized bilirubin from 7.4 mg/dL to 1.4 mg/dL



Significant improvement in reticulocytes from 25.5% to 2.73%



Sustained haptoglobin normalization



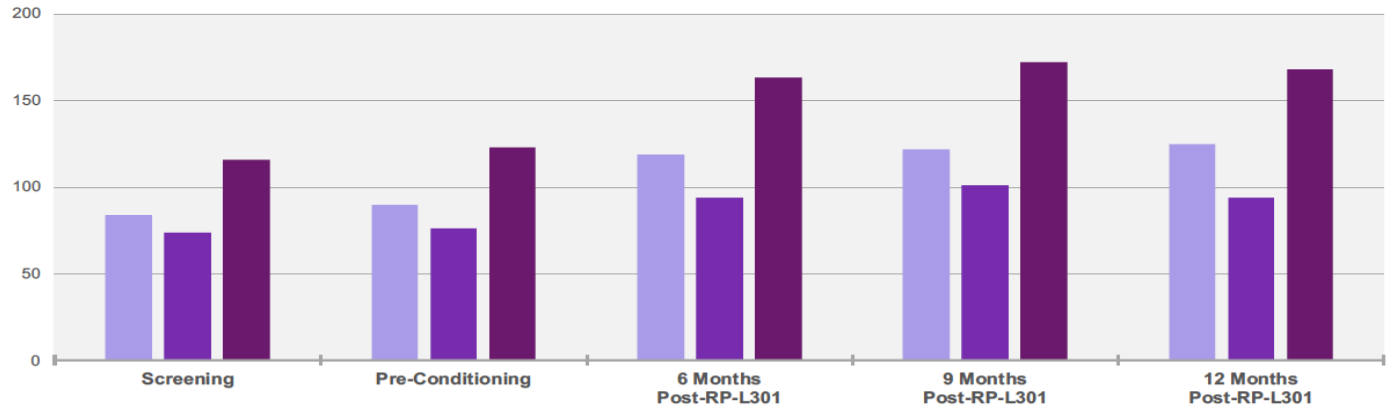
Significant decrease in erythropoietin levels

NOTE: Data cut-off as of November 3, 2021

Preliminary Results Indicate Improved Quality of Life During One Year Subsequent to RP-L301 therapy

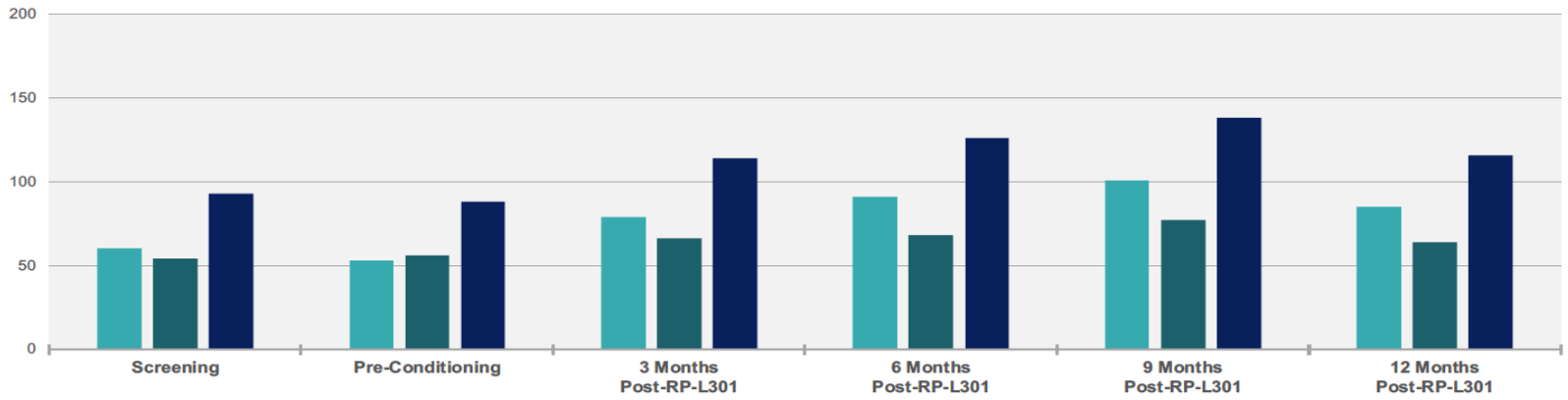
Functional Assessment of Cancer Therapy-Anemia (FACT-An)

L301-006-1001



L301-006-1001 FACT-An TOI Score
L301-006-1001 FACT-G Score
L301-006-1001 FACT-An Total Score

L301-001-1002



L301-001-1002 FACT-An TOI Score
L301-001-1002 FACT-G Score
L301-001-1002 FACT-An Total Score

Both patients have anecdotally reported improved quality of life (QoL) following RP-L301 administration; improved QoL has also been demonstrated by increase in FACT-An Total and component scores from baseline.

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RP-L301 Efficacy and Safety Summary

- **One-year follow-up in both initial adult patients indicates sustained efficacy:**
  - Doubling of pre-treatment baseline hemoglobin to normal-range sustained at 12M and substantial improvement in markers of hemolysis
  - Improvements in patient-reported QOL assessments
  - Transfusion independence post-treatment
- **Hematopoietic stem and progenitor cell collection appears safe and feasible in initial patients with severe PKD**
- **Safety profile of RP-L301 appears favorable**
  - Infusion well tolerated in (N=2); no IP-related serious adverse events (SAEs) at 12 months post- infusion in adult patients
  - Transient transaminase elevation seen in both subjects post conditioning and infusion with no clinical stigmata of liver injury; now resolving or resolved
  - Hematopoietic reconstitution occurred within 2 weeks post RP-L301 administration
  - Patients discharged from hospital within 1 month following RP-L301 infusion
- **Insertion site analysis for L301-006-1001 at 3 months post-RP-L301 in PB demonstrated a highly polyclonal pattern and no demonstrable insertions in proximity to potential oncogenic loci**
  - Additional ISA testing for both patients is ongoing

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Leukocyte Adhesion Deficiency-1: Interim Results of a Global Phase I/II Pivotal Phase II Study

Gaurav Shah, M.D.
Chief Executive Officer

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RP-L201 Clinical Trial Design & Outcomes Measures

Trial Design

- Non-Randomized Global Phase 1/2 Study (n=9)

Primary Outcomes

- **Phase 1:**
  - Safety & preliminary efficacy
- **Phase 2:**
  - Survival: proportion of patients alive at age 2 and at least 1-year post infusion (& not requiring alloHSCT)
  - Safety

Secondary Outcomes

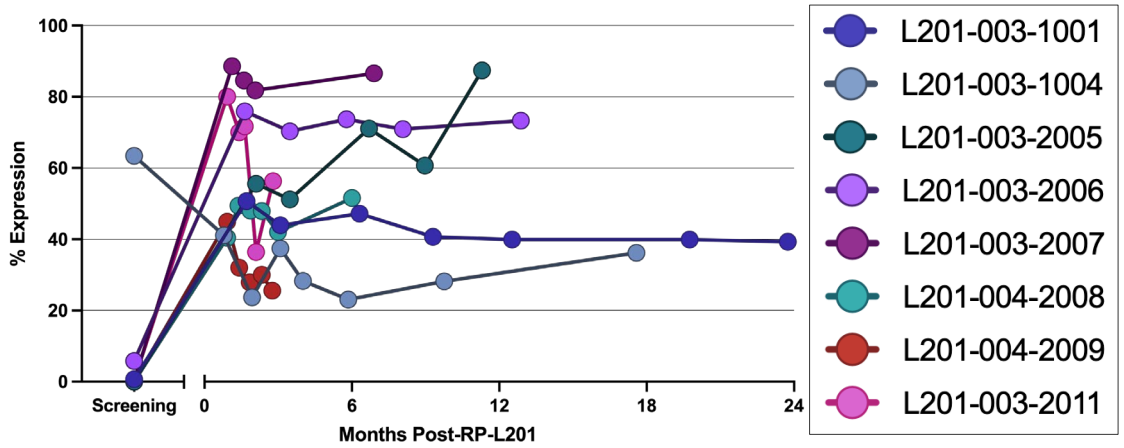
- **Incidence and severity of infections** (e.g., infection hospitalization-free survival, frequency of antimicrobial prophylaxis discontinuation)
- % of pts **w/neutrophil CD18 expression at least 10% of normal**
- % of pts w/neutrophil **VCN** of at least 0.1 copies/cell at 6m post-infusion
- Improvement/**normalization of neutrophilia**
- Resolution (partial or complete) of underlying **skin rash or periodontal abnormalities**

Patient	Sex	Age at enrollment	Drug Product VCN	CD34+ Cell Dose
L201-003-1001	F	9 yrs.	3.8	4.2×10^6 cells/kg
L201-003-1004	F	3 yrs.	2.5	2.8×10^6 cells/kg
L201-003-2005	F	3 yrs.	1.8	6.5×10^6 cells/kg
L201-003-2006	M	7 mo.	2.9	4.3×10^6 cells/kg
L201-003-2007	M	3 mo.	3.6	5.0×10^6 cells/kg
L201-004-2008	M	5 mo.	3.8	3.3×10^6 cells/kg
L201-004-2009	M	3 yrs.	2.0	4.5×10^6 cells/kg
L201-003-2011	F	2 yrs.	3.8	3.8×10^6 cells/kg
L201-002-2010*	F	4 yrs.	3.5	10×10^6 cells/kg

As of December 2021: Data reported from 8 of 9 patients; 3–24m follow-up. *Recent RP-L201 infusion

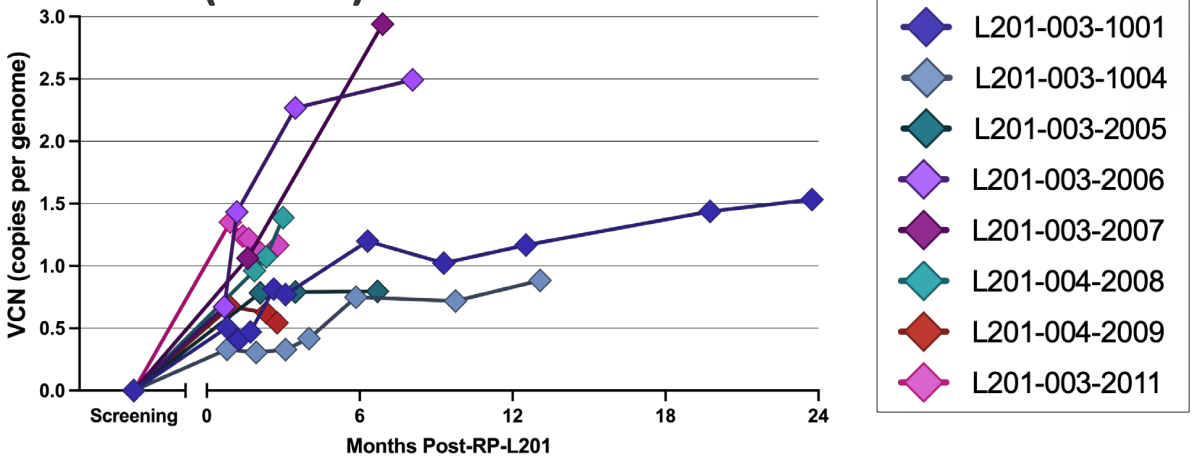
LAD-1: Clinical Efficacy Overview

% CD18 Expression (PMN) In Peripheral Blood

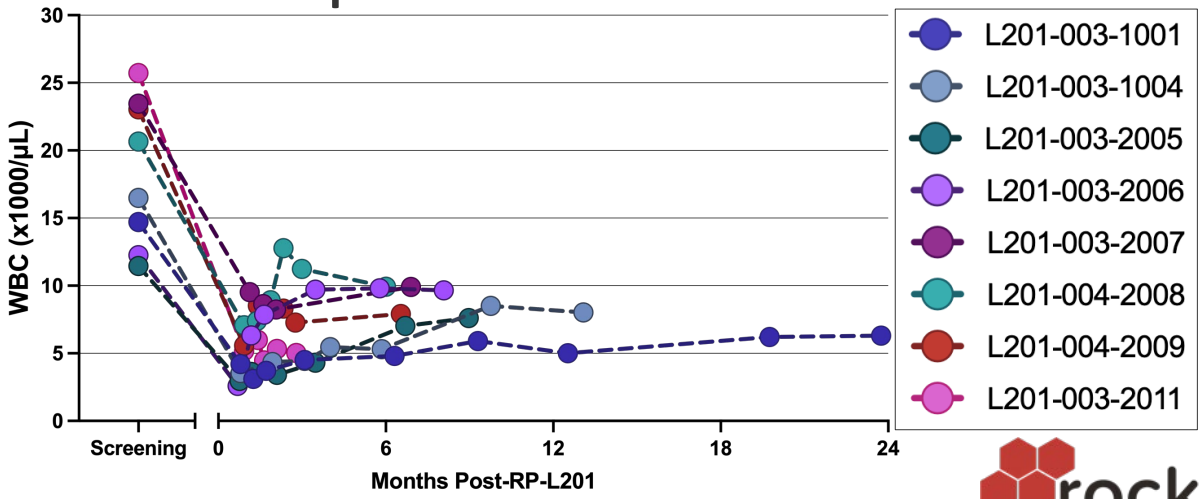


Dim/weak CD18 expression reported at baseline for subject L201-003-1004 in ~63% of cells

VCN (PBMC)



WBC in Peripheral Blood



- ***As of December 2021: Data reported in 8 of 9 patients with 3-24m follow-up***
- ***Final trial subject (patient 9 of 9) recently received RP-L201 infusion: L201-003-2010***
- ***Sustained %CD18 expression, VCN integration and leukocytosis resolution***

PMN: polymorphonuclear leukocytes

WBC: White Blood Cells

PBMC: peripheral blood mononuclear cell

RP-L201 Clinical Safety & Efficacy Summary

- Trial enrollment and dosing complete; 9 of 9 severe LAD-I patients successfully dosed with RP-L201.
  - Infusion has been **well tolerated** with no drug product-related SAEs.
  - Safety profile of RP-L201 appears favorable
- 8 of 9 patients have at least 3 months of follow-up with efficacy evident in **all 8** patients, including **4** patients with **≥ 12 months** of follow-up
  - **Patient L201-003-1001** with durable CD18 PMN expression ~40% at **24 months** and PB VCN of 1.53 copies/genome at **24 months** post-infusion
    - Resolution of pre-existing skin lesions, no infections/hospitalizations after hematopoietic reconstitution post RP-L201
  - **Sustained** >10% **CD18 PMN expression**, >0.1 copies/cell **VCN** integration and leukocytosis resolution across the cohort

Question & Answer Session

Gaurav Shah, M.D. Chief Executive Officer
Kinnari Patel, Pharm.D., M.B.A. Chief Operating Officer, President
Jonathan Schwartz, M.D. Chief Medical Officer, Senior Vice President

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