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 March 31, 2020, filed May 8, 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.
Gene Therapy: A Multi-Platform Approach

**In Vivo (In Body)**
AAV Gene Therapy

- Laboratory-produced AAV
- Direct intravenous injection
- Therapeutic AAV

**Ex Vivo (Outside Body)**
Lentiviral Gene Therapy

- Remove cells & isolate patient HSCs
- Laboratory-produced LV
- Therapeutic LVV
- Gene-modify HSCs
- Infusion of modified HSCs
### About Rocket Pharma

**Multi-Platform Gene Therapy (GTx) Company Targeting Rare Diseases**

1st-in-class with direct on-target mechanism of action (MOA) and clear clinical endpoints

<table>
<thead>
<tr>
<th>Ex-vivo Lentiviral vectors (LVV)</th>
<th>Fanconi Anemia (FA)</th>
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<tbody>
<tr>
<td></td>
<td>Leukocyte Adhesion Deficiency-I (LAD-I)</td>
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<td>Pyruvate Kinase Deficiency (PKD)</td>
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<td></td>
<td>Infantile Malignant Osteopetrosis (IMO)</td>
</tr>
<tr>
<td>Ex-vivo adeno-associated virus (AAV)</td>
<td>Danon Disease</td>
</tr>
</tbody>
</table>

### Multiple Near- & Medium-term Company Value Drivers

**Near-term Milestones (2020)**

- 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 (2021-2022)**

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

### Strong Precedents and World-Class Expertise

**Strong Precedents and Sound Strategy**

- Precedents for LVV- & AAV-based therapies
- Clearly-defined product metrics across indications
- Experienced company leadership
- Leading research and manufacturing partners
Rocket’s Leadership Team

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

**Raj Prabhakar, MBA**
Chief Business Officer & SVP
~17 years cell, gene and biotech business development

**Gayatri R. Rao, M.D., J.D.**
VP, Reg Policy & Patient Advocacy
7-Year Former Director of FDA’s Office of Orphan Products Development

**Kinnari Patel, Pharm.D., MBA**
COO & EVP, Development
*Led Opdivo and six rare disease indication approvals*

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

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

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

**Kamran Alam, CPA, MBA**
SVP, Finance and Principal Financial Officer
15+ years in biotech industry, including with AveXis

**Raj Prabhakar, MBA**
Chief Business Officer & SVP
~17 years cell, gene and biotech business development

**Gayatri R. Rao, M.D., J.D.**
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**Ramji Krishnan, Ph.D.**
VP, Manufacturing & Manufacturing Sciences
17+ years of product development and life cycle management expertise

**Brian C. Beard, Ph.D.**
AVP, CMC Lenti & AAV
15+ years cell and gene therapies expertise
## Rocket’s Expanding Pipeline: Potential for Significant Value Creation Near and Long Term

<table>
<thead>
<tr>
<th>Designations</th>
<th>Discovery</th>
<th>Preclinical</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>RP-A501</th>
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AAV LVV
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

- **Current available treatments**: Allogeneic hematopoietic stem cell transplant associated with 100-day mortality, GVHD, and additional increased cancer risk
- **Addressable Market**: Estimated U.S.+EU 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 U.S.; Advance Therapy Medicinal Product (ATMP) classification and PRIority MEdicines (PRIME) in the EU; Orphan Drug designation in the U.S./EU

---

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.

FA Path to Product Registration

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

Rocket-Sponsored FANCOLEN 1 Study

- Process B
  - 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
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)

HIU NJ 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
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 Somatic Mosaicism

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 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

* 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

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

Phase 1/2 Study of RP-L102 “Process A”

- No SAEs associated with the gene correction of FA HSPCs

- Infusion of corrected CD34\(^+\) cells in non-conditioned FA patients:
  - Progressive repopulation advantage of gene-corrected HSPCs at 1-3 years post-infusion (6/6 patients who were infused with >100,000 cCD34\(^+\) cells/kg)
  - Oligoclonal reconstitution and gene correction of multipotent HSCs
  - Progressive phenotypic correction of BM progenitors and PB T cells
  - At 1-3 years post-infusion, stabilization in PBC counts or overall increase in the erythroid lineage (two patients infused with the highest doses of corrected CD34\(^+\) cells)
RP-L102 “Process B” (FA) Clinical Trial

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

**Exclusion 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
- Available & eligible HLA-identical sibling donor
- Lansky PS ≤ 60%
- MDS or leukemia (including associated cytogenetic abnormalities)
- Mosaicism with stable/improved blood counts
# RP-L102: Subject Characteristics & Drug Product Metrics

## Subject Characteristics

<table>
<thead>
<tr>
<th>Subject</th>
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<th>1002</th>
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<tr>
<td>Age (y) Gender</td>
<td>5 F</td>
<td>6 F</td>
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<tr>
<td>WBC (/µL)</td>
<td>4,000</td>
<td>4,600</td>
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<tr>
<td>PMN (/µL)</td>
<td>1,280</td>
<td>1,340</td>
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<td>Hb (g/dL)</td>
<td>11.9</td>
<td>8.9</td>
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<tr>
<td>Plt (/µL)</td>
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<tr>
<th>Subject</th>
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<tr>
<td>MCV (fL)</td>
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<td>106.2</td>
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<td>BM34+ (/µL)</td>
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<td>34</td>
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<tr>
<td>FA mutation</td>
<td>c.2606A&gt;C, p.(Gln869Pro) c.2813A&gt;G, p.(Asp944Gly) c.3703C&gt;G, p.(Gln1235Glu)</td>
<td>c.?-1(522+1 523-1) del encompassing exons 1-5 c.?-1(283+1 284-1) del encompassing exons 1-3</td>
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## Investigational Product

<table>
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<tr>
<th>Subject</th>
<th>Nucleated Cells/kg</th>
<th>CD34+ Cells/kg^</th>
<th>CFCs/kg^</th>
<th>Mean VCN: Liquid Culture</th>
<th>Mean VCN: CFCs</th>
<th>CFC Survival MMC 10nM (%)</th>
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<tr>
<td>1001</td>
<td>7.8 x 10^6</td>
<td>2.0 x 10^5</td>
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<td>2.08</td>
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<td>1002</td>
<td>2.4 x 10^6</td>
<td>3.7 x 10^5</td>
<td>5.0 x 10^4</td>
<td>2.21</td>
<td>0.93^</td>
<td>47</td>
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* 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).
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

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.
RP-L102: Preliminary Clinical Data

**Blood Counts: Before and 6-Months After Receiving Therapy**

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

**Design**
- Enroll 10 pediatric patients globally

**Primary Outcomes**
- Phenotypic correction of bone marrow colony forming (progenitor) cells
  - MMC-resistance ≥10% at baseline 12-36 months post-infusion

**Secondary Outcomes**
- Phenotypic correction of T-lymphocytes in peripheral blood
  - DEB-induced chromosomal aberrations decrease from ≥50% at baseline to <50% 12-36 months post-infusion
- Engraftment of gene-corrected hematopoietic cells
  - At least 0.1 vector copy number/peripheral blood cell observed from 6 months post-infusion to the 3rd year post-infusion
- Prevention or rescue of bone marrow failure
  - Assessment of the need for treatment of bone marrow failure 6-36 months post-infusion

---

Overview:

• **Background**: Devastating multisystemic disorder caused by highly penetrant and X-linked dominant *LAMP2* mutations

• **Currently available treatments**: Non-curative heart transplants associated with considerable morbidity and mortality

• **Addressable Market**: Estimated U.S.+EU 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 & Fast Track designations in the U.S.
Danon Disease: An Impairment in Autophagy Caused by LAMP2B Mutations
RP-A501 Restores Cardiac Function in KO Mice

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

Cardiac Contractility

Cardiac Relaxation

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

Note: All mice were sacrificed at Month 10
RNA: RP-A501 Elicits Expression of hLAMP2B mRNA in Cardiac Tissue of KO Mice

*hlAMP2B = Human LAMP2B*
Protein: RP-A501 Elicits Durable Expression of LAMP2B Protein and Autophagic Flux in Heart

**Western Blot**

![Western Blot Image]

**LAMP2 Protein Expression**

![LAMP2 Protein Expression Graph]

**LC3-II Protein Expression**

![LC3-II Protein Expression Graph]

1. Data are Mean ± SEM. N=5-8 per group. Untx = Untreated, PBS = Phosphate buffered saline
2. Note: Mouse LAMP2 and Human LAMP2 data are from separate Western blots.
Structural: RP-A501 Reduces Autophagic Vacuoles in All Examined Organs

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Dose-dependent Widespread LAMP2 Expression in Cardiac Tissue

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

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<td>~10-fold higher transduction in cardiac vs. diaphragm; and comparable to other muscle</td>
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<tr>
<td>MPSIIIB</td>
<td>1 - 2E+13 vg/kg</td>
<td>NHP</td>
<td>≥10-fold higher transduction in cardiac vs. skeletal muscle in majority of animals</td>
<td>Nationwide Children’s</td>
<td>Murrey 2014</td>
</tr>
<tr>
<td>Non-specific</td>
<td>5E+10 vg/mouse</td>
<td>Mouse</td>
<td>5-10-fold higher transduction in cardiac vs. skeletal muscle</td>
<td>UNC</td>
<td>Pulicherla 2011</td>
</tr>
<tr>
<td>Pompe</td>
<td>4E+05 - 4E+08 vg/mouse</td>
<td>Mouse</td>
<td>~8-12-fold higher transduction in cardiac vs. skeletal muscle or diaphragm</td>
<td>U. Florida</td>
<td>Pacak 2006</td>
</tr>
<tr>
<td>SMA</td>
<td>2E14 vg/kg</td>
<td>Human</td>
<td>Heart VCN ~3-4, Muscle &amp; CNS ~1</td>
<td>AveXis</td>
<td>Kaspar 2019 (ASGCT 2019)</td>
</tr>
</tbody>
</table>
VCN in Non-Human Primates at Day 102 High in Cardiac Tissues

Differential distribution of vector genomes was observed, with highest levels seen in liver followed by heart.

**VCN in NHPs Dosed with 3x10^{14} vg/kg**

<table>
<thead>
<tr>
<th>Tissue Type</th>
<th>NHP ID 366</th>
<th>NHP ID 690</th>
<th>NHP ID 2750</th>
<th>NHP ID 4247</th>
</tr>
</thead>
<tbody>
<tr>
<td>Brain Cerebellum</td>
<td>0.12</td>
<td>0.06</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Brain Frontal</td>
<td>1.65</td>
<td>0.63</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Brain Hipp.</td>
<td>0.50</td>
<td>0.27</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Brain Medulla</td>
<td>12.26</td>
<td>1.34</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Brain Occ. Cortex</td>
<td>0.73</td>
<td>0.09</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Brain Parietal</td>
<td>0.35</td>
<td>0.50</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Brain Temporal</td>
<td>0.59</td>
<td>0.48</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Diaphragm</td>
<td>3.25</td>
<td>1.03</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>EYE</td>
<td>0.03</td>
<td>0.56</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Heart LA</td>
<td>35.74</td>
<td>58.07</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Heart LV</td>
<td>8.41</td>
<td>11.90</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Heart RA</td>
<td>57.57</td>
<td>201.58</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Heart RV</td>
<td>10.82</td>
<td>19.76</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Kidney Left</td>
<td>4.71</td>
<td>1.55</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Kidney Right</td>
<td>5.83</td>
<td>1.70</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Liver Caudate</td>
<td>2536.51</td>
<td>2373.70</td>
<td>0.02</td>
<td>0.00</td>
</tr>
<tr>
<td>Liver Left Lobe</td>
<td>2334.43</td>
<td>1862.57</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Liver Middle Lobe</td>
<td>2447.59</td>
<td>2010.33</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Liver Right Lobe</td>
<td>2248.60</td>
<td>2168.30</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Lung Left</td>
<td>4.82</td>
<td>4.93</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Lung Right</td>
<td>6.74</td>
<td>5.17</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Lymph Node Inguinal</td>
<td>19.01</td>
<td>10.01</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Lymph Node Mand.</td>
<td>8.25</td>
<td>7.60</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Lymph Node Mesen.</td>
<td>1.91</td>
<td>0.87</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Muscle Gastroc.</td>
<td>0.07</td>
<td>0.52</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Muscle Quad.</td>
<td>0.61</td>
<td>0.28</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Pancreas</td>
<td>1.11</td>
<td>1.69</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Spleen</td>
<td>2.54</td>
<td>1.96</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Testes Left</td>
<td>1.16</td>
<td>0.24</td>
<td>0.00</td>
<td>0.00</td>
</tr>
<tr>
<td>Testes Right</td>
<td>0.94</td>
<td>0.27</td>
<td>0.00</td>
<td>0.00</td>
</tr>
</tbody>
</table>

- 30 mg tissue
- 20 ng DNA template
- Primer/probe to WPRE
- qPCR (40 cycles)
Protein Expression in Non-Human Primates Highest in Cardiac Tissues

Western Blot Analysis

LAMP2 Assessment Based on Total Protein\(^1\) Loaded on Gel

- Higher levels of transgenic human LAMP2 protein detected over endogenous NHP LAMP2 in most tissues tested, specifically the heart

\(^1\)Normalized to total protein instead of GAPDH, as housekeeping protein levels were variable.
Summary of Preclinical Data

• Shows Phenotypic Improvements as Low as 5e13 vg/kg:
  - Survival benefit at higher doses
  - Dose-dependent restoration of cardiac function
  - Improvement in liver enzymes

• 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 include high concentrations in heart tissue (for NHP, ~10x higher on average than in skeletal muscle and CNS)
Design
• Enroll ~12-24 pediatric and young adult male patients
• Two dose levels investigated in 4 distinct cohorts (n=3-6 patients)
  - Cohort 1: Adult and age 15 and older: Low Dose
  - Cohort 2: Adult and age 15 and older: High Dose
  - Cohort 3: Pediatric age 8-14: Low Dose
  - Cohort 4: Pediatric age 8-14: High Dose

Primary Outcomes
• Evaluation and assessment of safety at both dose levels
• Assessment of target tissue transduction
• Assessment of effect on cardiomyocyte histology
• Assessment of clinical stabilization or improvement via cardiac imaging, serology and exercise testing

Status
• Moving to Higher Dose Adult Cohort. No DLTs in Cohort 1 (Low Dose)

Source: https://clinicaltrials.gov/ct2/show/NCT03882437?cond=danon&rank=2
Danon Disease Prevalence: ~15-30K in the U.S.+EU

Hypertrophic Cardiomyopathy (HCM)
- U.S. HCM Prevalence: 600K-1MM+ \(^1\)
- 1-4% of HCM patients consistently identified with \(LAMP2\) mutations in multiple studies with >1000 subjects evaluated\(^2\)
- Danon Disease Patients with HCM\(^3\)
  - 85% of males
  - 30% of females

Dilated Cardiomyopathy (DCM)
- Danon Disease Patients with DCM\(^3\)
  - 15% of males
  - 50% of females

\(^1\)Source: J Am Coll Cardiol. 2015 Mar 31;65(12):1249-1254.
## Danon Disease Causes 1-4% of Hypertrophic Cardiomyopathy: Consistent Presence in Multiple Series Published 2004-Present

<table>
<thead>
<tr>
<th>Author &amp; Year</th>
<th>Age</th>
<th>n HCM</th>
<th>n Danon</th>
<th>% Danon</th>
<th>Note</th>
</tr>
</thead>
<tbody>
<tr>
<td>Charron 2004</td>
<td>N.A.</td>
<td>197</td>
<td>2</td>
<td>1.0%</td>
<td>Studied LAMP2 mutations in 197 HCM patients at a general hospital in Paris</td>
</tr>
<tr>
<td>Arad 2005</td>
<td>12-75</td>
<td>75</td>
<td>2</td>
<td>2.7%</td>
<td>Studied glycogen storage diseases in 75 consecutive pts diagnosed with HCM (multicenter U.S./EU). No cases of Pompe or Fabry were detected.</td>
</tr>
<tr>
<td>Yang 2005</td>
<td>1m-15y</td>
<td>50</td>
<td>2</td>
<td>4.0%</td>
<td>Studied LAMP2 mutations in 50 pts with ped./juvenile onset HCM (single U.S. center). Additional DD identified in relatives of the n=2 probands were not included in this analysis.</td>
</tr>
<tr>
<td>Cheng 2012</td>
<td>N.A.</td>
<td>50</td>
<td>3</td>
<td>2.3%</td>
<td>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.</td>
</tr>
</tbody>
</table>

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

**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 GVHD
- **Addressable Market:** Estimated 25-50 pts treatable per year for severe population; up to 100 for potential expansion into moderate population in the U.S.+EU 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.; Advance Therapy Medicinal Product (ATMP) classification in EU; Orphan Drug designation in the U.S./EU

---

1 Defective expression of β2 integrin on leukocytes limits their extravasation to inflamed sites.
## LAD-I Program Summary

### Ultra-rare Disease = Streamlined Regulatory Approach

<table>
<thead>
<tr>
<th>Potential design &amp; clinical endpoints</th>
<th>Target Patient Population: Severe LAD-I patients (CD18&lt;2%), ~2/3 mortality by 2y</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td><strong>Control:</strong> Literature review of ~300 pts. (Rocket/academic collaborative publication&lt;sup&gt;1&lt;/sup&gt;)</td>
</tr>
<tr>
<td></td>
<td><strong>Potential Clinical Endpoints:</strong> Modest correction of CD18 expression, survival</td>
</tr>
</tbody>
</table>

### Efficacy Trials & Registration Status – Ahead of Schedule

<table>
<thead>
<tr>
<th>Registration &amp; study planning on-schedule</th>
<th>✓ Orphan Drug (U.S./EU) and Pediatric Rare Disease (U.S.) designations granted</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>✓ IND &amp; Phase 1/2 cleared by FDA</td>
</tr>
<tr>
<td></td>
<td>✓ Spain IMPD cleared</td>
</tr>
<tr>
<td></td>
<td>✓ U.S. PI (UCLA Dr. Don Kohn)</td>
</tr>
<tr>
<td></td>
<td>✓ Recruitment underway from around the globe</td>
</tr>
<tr>
<td></td>
<td>❑ 3 global sites planned in the U.S./EU</td>
</tr>
</tbody>
</table>

### Product/Manufacturing Optimization

| Process now optimized | ✓ VCN using GMP vector with transduction enhancers consistently ~3 (Target VCN >1) |

---

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

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

Kaplan-Meier Survival Estimates by Neutrophil CD18 Expression
-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.

Poster Presentation at ASGCT May 2018

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)

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

**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 (U.S. and EU)

**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

Medical History of Patient L-201-003-1001

Historical patient records collected by UCLA Mattel Children’s Hospital
LAD has received CIRM Funding
**Key Drug Product Metrics**

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

**Clinical Results**

- CD18 Expression in Peripheral Blood:
  - 3-month CD18 expression post-treatment: 45%
  - 6-month CD18 expression post-treatment: 47%
  - Pre-treatment CD18 expression was <1%

---

%CD18 Expression in Peripheral Blood

%CD11a Expression in Peripheral Blood

%CD11b Expression in Peripheral Blood
RP-L201: Flow Cytometry 6-Months Post-Treatment

Healthy Donor Control

L201-003-1001

**Neutrophils**

CD18 Expression

- **Healthy Donor Control**: 99%
- **L201-003-1001**: 47%

UCL Gosh Data Presented May 2020
LAD has received CIRM Funding
RP-L201: Visible Improvements Post-Treatment

Spontaneous Abdominal Lesion

Baseline (Pre-Treatment)  3-months (Post-Treatment)  6-months (Post-Treatment)
Prior to Gene Therapy: BM Bx Site

BM Bx Site 2 Days After 3-Month Marrow Bx

Lower Back Lesion (after BM aspirate)

Lower Back (after BM aspirate)

RP-L201: Visible Improvements Post-Treatment
No Infection/Inflammation After 3-Month Bone Marrow Biopsy
Pyruvate Kinase Deficiency (PKD)
Monogenic Red Blood Cell Hemolytic Disorder

Overview:

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

- **Addressable Market**: ~250-500 patients/year

- **RP-L301**: Corrects multiple components in a PKD mouse model, including increases in hemoglobin, reduction in reticulocytosis, correction of splenomegaly and reduction in hepatic erythroid clusters and iron deposits

- **Regulatory Designations**: Fast Track in the U.S. and Orphan Drug designation in the U.S./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 U.S./EU of approximately 3,000 to 8,000.
PKD correction observed when at least 20-30% of bone marrow cells are genetically corrected.

PKD correction was achieved when ≥0.3 copies of the vector were detected in peripheral blood mononuclear cell populations.

Spleen size and weight correlated to vector copy number.

Mouse Model Indicates Correlation Between Genetic Correction and Reversal of Hemolytic Phenotype Including Normalization of Splenomegaly.
<table>
<thead>
<tr>
<th>Product/Manufacturing Optimization</th>
</tr>
</thead>
</table>
| **Positive outlook for near term optimization PoC** | • Target engraftment of 20-30%  
  • Optimization of vector manufacturing + transduction process  
  • VCN now 2-4 range with TDx Enhancers |

<table>
<thead>
<tr>
<th>Clinical Efficacy/Registration Status</th>
</tr>
</thead>
</table>
| **Registration & study planning on-schedule** | ✓ Registry efforts underway  
  ✓ U.S. site identified as Stanford University  
  ❑ Plan to treat 2 adults, then 2 older and then 2 younger pediatric patients  
  ❑ 18 post-splenectomy, transfusion-dependent patients pre-identified in EU |
RP-L301 Addressable Market: Approximately 250-500 Patients per Year

• Published Prevalence:
  - PKD in non-Hispanic Caucasians calculated to be 51 per million\(^1\)
  - Conservative estimates conclude a number from 3,000 to 8,000 in the U.S.+EU combined

• Addressable PKD market estimated to be between 250-500 patients per year in the U.S.+EU

• ~50% non-response rate reported in one targeted therapy in development\(^2\)

---

\(^2\)https://www.sec.gov/Archives/edgar/data/1439222/000119312517366278/d443156dex991.htm
RP-L301 (PKD) Clinical Trial and Outcome Measures

Design
- Enroll 6 patients globally, who have a history of severe transfusion dependent anemia
  - Adult cohort (n=2)
  - Pediatric patients ages 12-17 (n=2)
  - Pediatric patients ages 8-11 (n=2)
    - Pediatric patient dosing to commence after determining safety in adult cohort

Primary Outcomes
- Safety associated with treatment

Secondary Outcomes
- Multi-lineage gene correction in peripheral blood and bone marrow
- Reduction in transfusion dependence and/or transfusion requirements
- Reduction in anemia and hemolysis

1Source: https://clinicaltrials.gov/ct2/show/NCT04105166?term=Rocket&cond=Pyruvate+Kinase+Deficiency&rank=1
Infantile Malignant Osteopetrosis (IMO)
Monogenic bone resorption disorder

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 before age 10

• **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

• **Regulatory Designations:** Rare Pediatric Disease and Orphan Drug designations in the U.S.

---

\(^1\)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
# Growing IP Portfolio

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

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

## Rocket Proprietary Filed IP

<table>
<thead>
<tr>
<th>Extensive patent portfolio across multiple platforms</th>
<th>Multiple pending patent applications for ex-vivo LVV programs</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Multiple pending patent applications for in-vivo AAV</td>
</tr>
</tbody>
</table>
World-Class Research and Development Partners

- CIBER
- CIEMAT
- Fred Hutchinson Cancer Research Center
- IIS FJD
- Lund University
- Memorial Sloan Kettering Cancer Center
- REGENXBIO
- Stanford Medical School
- University of California, San Diego
- University of California, Los Angeles
Expansion into Cranbury, NJ: R&D/CMC Efforts and Eventual cGMP Manufacturing

2019
- Secured adequate supply of cGMP AAV9 to commercialization in partnership with CMO
- Established an agreed path forward with Agency using current process

2020
- 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)
- Planned one-time additional spend of ~$20-30M in 1H’20 dedicated to manufacturing build, and normal spend thereafter
- Initiating AAV tech transfer activities, projected GMP clinical product release in 2021
- Enables dual-sourcing for Danon commercial capacity
Near and Long-Term Value Drivers

Potential for Five Gene Therapy Products to be Approved by 2025

- Danon: Endorsed to Advance to Next Cohort
- LAD-I: Phase 1 Data Update (first patient)
- FA: Additional “Process A” Data Update
- Danon: First Patient Treatment in Higher Dose Cohort
- PKD: First Patient Treatment
- LAD-I: Phase 1 Data Update
- FA: Preliminary “Process B” Data
- LAD-I: Initiate Phase 2 Study
- PKD: Preliminary Phase 1 Data
- IMO: Initiation of Clinical Study
- Danon: Preliminary Phase 1 Data

Danon Day: The health and safety of our patients and families is our utmost priority, as a result of COVID-19 Danon Day has been postponed.