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 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 Annual Report on Form 10-K for the year ended December 31, 2018. 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
- Therapeutic AAV
- Direct intravenous injection

**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>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Leukocyte Adhesion Deficiency-I (LAD-I)</td>
</tr>
<tr>
<td></td>
<td>Pyruvate Kinase Deficiency (PKD)</td>
</tr>
<tr>
<td></td>
<td>Infantile Malignant Osteopetrosis (IMO)</td>
</tr>
</tbody>
</table>

| In-vivo adeno-associated virus (AAV) | Danon Disease |

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

**Near-term Milestones (2019)**

- Four programs in the clinic (FA, LAD-I, PKD, Danon)
- Additional clinical data for FA and LAD-I (Next 12-18 months)
- FA and LAD-I advance to potential registration trial stage

**Medium-term Milestones (2020-2021)**

- Registrational studies ongoing in four programs
- First global submission (BLA/MAA)
- Platform establishment and pipeline expansion
- Currently planned 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
# Leadership Team: Expertise in GTx & Rare Diseases Clinical Development

<table>
<thead>
<tr>
<th>Name</th>
<th>Title</th>
<th>Background/Expertise</th>
</tr>
</thead>
<tbody>
<tr>
<td>Gaurav Shah, M.D.</td>
<td>President &amp; Chief Executive Officer</td>
<td>~20 years cell and gene therapy development &amp; manufacturing</td>
</tr>
<tr>
<td>Jonathan Schwartz, M.D.</td>
<td>CMO &amp; SVP, Clinical Development</td>
<td>Led multiple biologics approvals</td>
</tr>
<tr>
<td>Kinnari Patel, Pharm.D., MBA</td>
<td>COO &amp; EVP, Development</td>
<td>Led Opdivo and six rare disease indication approvals</td>
</tr>
<tr>
<td>Annahita Keravala, Ph.D.</td>
<td>AVP, AAV Platform</td>
<td>20+ years gene therapy expertise</td>
</tr>
<tr>
<td>Gayatri R. Rao, M.D., J.D.</td>
<td>VP, Reg Policy &amp; Patient Advocacy</td>
<td>7-Year Former Director of FDA’s Office of Orphan Products Development</td>
</tr>
<tr>
<td>Raj Prabhakar, MBA</td>
<td>SVP, Bus Operations &amp; Bus Development</td>
<td>~17 years cell, gene and biotech business development</td>
</tr>
<tr>
<td>Claudine Prowse, Ph.D.</td>
<td>SVP, Strategy &amp; Corporate Dev</td>
<td>~20 years capital markets, strategy, corporate development</td>
</tr>
<tr>
<td>Christopher Ballas, Ph.D.</td>
<td>VP, Manufacturing</td>
<td>~20 years cell and gene therapy development &amp; manufacturing</td>
</tr>
<tr>
<td>Brian C. Beard, Ph.D.</td>
<td>AVP, CMC Lenti &amp; AAV</td>
<td>15+ years cell and gene therapies expertise</td>
</tr>
</tbody>
</table>

- **Harvard University**
- **Lilly**
- **Novartis**
- **Mount Sinai**
- **Columbia University**
- **Beckman Coulter**
- **Biogen**
- **Geisinger**

**Leadership Team Expertise:**

- **GTx & Rare Diseases Clinical Development**
- **Spearheaded Kymriah (CART-19) development at Novartis towards approval**
- **Led multiple biologics approvals**
- **Led Opdivo and six rare disease indication approvals**
- **~20 years cell and gene therapy development & manufacturing**
- **~17 years cell, gene and biotech business development**
- **~20 years capital markets, strategy, corporate development**
- **~20 years cell and gene therapy development & manufacturing**
- **15+ years cell and gene therapies expertise**
## Rocket’s Expanding Pipeline: Potential for Significant Value Creation Near and Long Term

<table>
<thead>
<tr>
<th>Discovery</th>
<th>Preclinical</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Designations</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>RP-A501</strong>&lt;br&gt;Danon Disease</td>
<td>*</td>
<td>*</td>
<td>Process A (E.U.)*&lt;br&gt;Process B (E.U.)&lt;br&gt;Process B (U.S.)</td>
<td>Fast Track, Orphan Drug (U.S.)</td>
</tr>
<tr>
<td><strong>RP-L102</strong>&lt;br&gt;Fanconi Anemia</td>
<td>*</td>
<td>*</td>
<td>*</td>
<td>RMAT, ATMP, Fast Track, Rare Pediatric, Orphan Drug (U.S./E.U.)</td>
</tr>
<tr>
<td><strong>RP-L201</strong>&lt;br&gt;Leukocyte Adhesion Deficiency-I</td>
<td>*</td>
<td>*</td>
<td>*</td>
<td>ATMP, Fast Track, Rare Pediatric, Orphan Drug (U.S./E.U.)</td>
</tr>
<tr>
<td><strong>RP-L301</strong>&lt;br&gt;Pyruvate Kinase Deficiency</td>
<td>*</td>
<td>*</td>
<td>*</td>
<td>Orphan Drug (U.S./E.U.)</td>
</tr>
<tr>
<td><strong>RP-L401</strong>&lt;br&gt;Infantile Malignant Osteopetrosis</td>
<td>*</td>
<td>*</td>
<td>*</td>
<td>Rare Pediatric, Orphan Drug (U.S.)</td>
</tr>
</tbody>
</table>

*Phase 1/2

**AAV**<br>**LVV**
Danon Disease
Monogenic Heart Failure Syndrome

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 US+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 US
Danon Disease: An Impairment in Autophagy Caused by \textit{LAMP2B} Mutations

Macroautophagy

\begin{itemize}
\item Isolation Membrane
\item Cytosolic Proteins and Organelles
\item Autophagosome
\item Autolysosome
\item Acid Hydrolases
\item Lysosome
\item LAMP2
\item Danon Disease
\item X-linked cardiac & skeletal myopathy
\end{itemize}
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

LAMP2 Protein Expression

LC3-II Protein Expression

Note: Mouse LAMP2 and Human LAMP2 data are from separate Western blots.

1Data are Mean ± SEM. N=5-8 per group. Untx = Untreated, PBS = Phosphate buffered saline

12
Structural: RP-A501 Reduces Autophagic Vacuoles in All Examined Organs

<table>
<thead>
<tr>
<th></th>
<th>LAMP2 KO</th>
<th>AAV9.LAMP2B</th>
</tr>
</thead>
<tbody>
<tr>
<td>Wild Type</td>
<td>KO Control</td>
<td>5e13 vg/kg</td>
</tr>
<tr>
<td>Heart</td>
<td>Heart images</td>
<td>images</td>
</tr>
<tr>
<td>Liver</td>
<td>Liver images</td>
<td>images</td>
</tr>
<tr>
<td>Skeletal Muscle</td>
<td>Skeletal Muscle images</td>
<td>images</td>
</tr>
</tbody>
</table>
Dose-dependent Widespread LAMP2 Expression in Cardiac Tissue

<table>
<thead>
<tr>
<th></th>
<th>Wild Type</th>
<th>PBS</th>
<th>1e13 vg/kg</th>
<th>5e13 vg/kg</th>
<th>1e14 vg/kg</th>
<th>2e14 vg/kg</th>
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<tbody>
<tr>
<td>hLAMP2</td>
<td></td>
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<tr>
<td>LAMP2 KO</td>
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<td>AAV9.LAMP2B</td>
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<tr>
<td>LAMP2Dys/DAP</td>
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</table>
AAV9 Vector Shows Consistent & Strong Cardiac Tropism in Several Studies Across Different Species

<table>
<thead>
<tr>
<th>Disorder &amp; Vector</th>
<th>Dose</th>
<th>Species</th>
<th>Results</th>
<th>Sponsor</th>
<th>Reference</th>
</tr>
</thead>
<tbody>
<tr>
<td>LGMD2A AAV9.hCAPN3</td>
<td>3E+13 vg/kg</td>
<td>NHP</td>
<td>8-80-fold higher transduction in cardiac vs. skeletal muscle</td>
<td>Genethon</td>
<td>Lostal (ASGCT 2018)</td>
</tr>
<tr>
<td>Non-specific AAV9.Luc</td>
<td>3E+12 vg/kg</td>
<td>NHP</td>
<td>~ 10-fold higher transduction in cardiac vs. diaphragm; and comparable to other muscle</td>
<td>UNC</td>
<td>Tarantal 2016</td>
</tr>
<tr>
<td>Pompe AAV9.hGAA</td>
<td>1E+11 vg/mouse</td>
<td>Mouse</td>
<td>~ 10-fold higher transduction in cardiac vs. diaphragm</td>
<td>U. Florida</td>
<td>Falk 2015</td>
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<tr>
<td>DMD AAV9.μDys</td>
<td>1.9 - 6.2E+14 vg/kg</td>
<td>Dog</td>
<td>2-3 fold higher transduction in cardiac vs. skeletal muscle</td>
<td>U. Missouri</td>
<td>Yue 2015</td>
</tr>
<tr>
<td>SMA AAV9.SMN</td>
<td>3E+14 vg/kg &amp; 1E+13 vg/kg Mouse &amp; NHP</td>
<td>~ 100-fold higher transduction in cardiac vs. skeletal muscle (mouse)</td>
<td>Nationwide Children’s</td>
<td>Meyer 2014</td>
<td></td>
</tr>
<tr>
<td>MPSIIB AAV9.hNAGLU</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 AAV9.Luc</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 AAV9.hGAA</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 AAV9.SMN</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.

- 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

GAPDH (housekeeping gene)

LAMP2

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\(^1\)
- 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 Endpoints\(^1\)
- 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

\(^1\)Source: https://clinicaltrials.gov/ct2/show/NCT03882437?cond=danon&rank=2
RP-A501 Clinical Development Plans

2019
- U.S. Phase 1 Study with clinical GMP AAV9 RP-A501 in patients with Danon disease
- Continue registry & patient education/identification
- Clinical retrospective database in progress
- Prospective natural history study ongoing¹

2020
- Phase 2/Registration-enabling Study for global submission seeking Accelerated Approval

¹Natural History ClinicalTrials.gov Identifier: NCT03766386
Danon Disease Prevalence: ~15-30K in the US+EU

Hypertrophic Cardiomyopathy (HCM)
- US 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 US/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 US 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>

Fanconi Anemia (FA)
Monogenic DNA-repair disorder

**RP-A501**
Danon Disease

**RP-L102**
Fanconi Anemia

**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**\(^2\): Estimated US+EU target population of approximately 2,000 patients, 400-500 patients/year

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

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

---

\(^1\) Alter Br J Hametol 2010.
\(^2\) CIBMTR and EBMT registries 2009-2013.
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

Optimization

Rocket-Sponsored Process B

(Higher cell doses, transduction enhancers, commercial-grade vector and modified cell processing)

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

BLA/MAA
Updated Data from Phase 1/2 Gene Therapy Trial of RP-L102 in Patients with Fanconi Anemia

Key Efficacy Measurements:

- **Genetic correction of bone marrow cells (engraftment):** measured by peripheral blood VCN

- **Functional and phenotypic correction of bone marrow cells:** measured by resistance to mitomycin-C (MMC)

- **Functional and phenotypic correction of blood cells:** measured by chromosomal stability of T-lymphocytes in the presence of diepoxybutane (DEB)

- **Hematologic correction:** measured by changes in previously declining pre-treatment blood count trajectories
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)

Ciemat Data Presented at ASGCT May 2019

*Minimally Acceptable Dose

cCFU = Corrected Colony Forming Units; pVCN: Product VCN
Functional Correction of Bone Marrow

 MMC assay identifies cells resistant to Mitomycin-C (MMC), a DNA damaging agent toxic to (uncorrected) FA blood and bone marrow cells.
Gene Therapy Confers a Phenotype Similar to Somatic Mosaicism

Ciemat Data Presented at ASGCT May 2019
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

Ciemat Data Presented at ASGCT May 2019
Gene Therapy Stabilizes Previously Declining Blood Counts. Most Encouraging Stability When BM Gene Correction Exceeds 50%*

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

Ciemat Data Presented at ASGCT May 2019
• **Fanconi anemia occurs in one in every 160,000 individuals worldwide**\(^1\)
  - Most commonly inherited bone marrow failure syndrome\(^2\)
  - Approximately one in every 181 people in the US is a carrier of Fanconi Anemia\(^3\)
  - More common among people of Ashkenazi Jewish descent, the Roma population of Spain, and black South Africans.\(^1\)

• **Fanconi anemia incidence:**
  - Approximately one in every 130,000 births in the US\(^3\)

• **30-40% of patients undergo HSCT**\(^2\)

---

\(^1\)Source: https://ghr.nlm.nih.gov/condition/fanconi-anemia#statistics
\(^2\)Source: Haematologica. 2018 Jan;103(1):30-39
\(^3\)Source: https://www.stjude.org/disease/fanconi-anemia.html
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 US+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 US; Advance Therapy Medicinal Product (ATMP) classification in EU; Orphan Drug designation in the US/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:</th>
<th>~2/3 mortality by 2y</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Control:</td>
<td>Lit review of ~300 pts. (Rocket/academic collaborative publication)</td>
</tr>
<tr>
<td></td>
<td>Potential Clinical Endpoints:</td>
<td>Modest correction of CD18 expression, Survival</td>
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</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./E.U.) 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>✓ US PI (UCLA Dr. Don Kohn)</td>
</tr>
<tr>
<td></td>
<td>□ 3 global sites planned in the US/EU</td>
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<tr>
<td></td>
<td>□ Recruitment underway from around the globe</td>
</tr>
</tbody>
</table>

## Product/Manufacturing Optimization

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

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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 Elicits Dose-Dependent CD18 Expression & Phenotypic Correction in KO Mice

VCN and CD18 Expression in Peripheral Blood Cells

Preferential Migration of Corrected Neutrophils

- Likely 5-10% neutrophil CD18 expression is sufficient to enable phenotypic reversal in severe LAD-I

Ciemat Poster Presentation at ASGCT May 2019

1 MOI-multiplicity of infection; CD18 measured by flow cytometry; VCN determined by qPCR
LAD-I: Improved Process Produces VCN >~2-4

VCN in Liquid Culture

![Bar chart comparing VCN production in liquid culture between old and improved processes.](image)

- **Old Process**: Using GMP vector batch, VCN production is shown as a function of MOI (multiplicity of infection).
- **Improved Process**: Shows enhanced VCN production compared to the old process.

Source: Company data on file
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**: Orphan Drug designation in the US/EU

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1. One glucose molecule is metabolized into two Phosphoenolpyruvate and ultimately two Pyruvate (pyruvic acid) molecules; this final enzymatic step yields two additional ATPs from each glucose molecule.
2. Market research indicates the application of gene therapy to broader populations could increase the annual market opportunity from approximately 250 to 500, based on an estimated prevalence in the US/EU of approximately 3,000 to 8,000.
Mouse Model Indicates Correlation Between Genetic Correction and Reversal of Hemolytic Phenotype Including Normalization of Splenomegaly

- 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

Ciemat Data Presented at ASGCT May 2019
**PKD Program Summary**

### Product/Manufacturing Optimization

**Positive outlook for near term optimization PoC**

- Target engraftment of 30-40%
- Optimization of vector manufacturing + transduction process
- VCN now 2-4 range with TDx Enhancers

### Clinical Efficacy/Registration Status

**Registration & study planning on-schedule**

- Registry efforts underway
- US 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
RPL301 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 US+EU combined

- **Addressable PKD market estimated to be between 250-500 patients per year in the US+EU**

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

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\(^2\)https://www.sec.gov/Archives/edgar/data/1439222/000119312517366278/d443156dex991.htm
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 US

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\(^1\)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
### 5 in-licensed patent families for GTx products and related tech

#### Supporting current pipeline efforts
- In-licensed four pending international patent applications filed under Patent Cooperation Treaty (PCT):
  - FA (2)
  - LAD-I
  - PKD
- One pending PCT application:
  - Danon (Licensed through UCSD with worldwide rights to AAV9 via REGENXBIO collaboration)

#### Efforts underway to protect and enhance proprietary technology
- Additional pending patent applications in the US, Europe and Japan relating to devices, methods, and kits for harvesting and genetically modifying target cells
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
Near and Long-Term Value Drivers
Potential for Five Gene Therapy Products to be Approved by 2025

2Q19

- FA (RP-L102): Updated Data from Four Patients Treated Under “Process A”
- Danon (RP-A501): FPI for Phase 1 Study
- LAD-I (RP-L201): FPI for Registration-enabling Phase 1/2 Study

2H19

- FA (RP-L102): Initial Phase 1 Data Under “Process B”
- FA (RP-L102): Regulatory Alignment on Final Endpoints for Registration
- LAD-I (RP-L201): Initial Phase 1 Data
- PKD (RP-L301): FPI for Phase 1 Study

2020

- Additional Data from FA (RP-L102) and LAD-I (RP-L201) Studies
- Danon (RP-A501): Phase 1 Data
- Danon (RP-A501): Initiate Phase 2/Registration-enabling Study
- PKD (RP-L301): Phase 1 Data
- IMO (RP-L401): Initiate Clinical Study