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) and Infantile Malignant Osteopetrosis (IMO), 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, Rocket’s ability to commence a registrational study in FA within the projected time periods, 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 Annual Report on Form 10-K for the year ended December 31, 2017. 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.
Developing best-in-class gene and cell therapies for patients with devastating diseases.

Inspired by transformative innovation, built on a sustainable and integrated multi-platform approach.
### 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

| Ex-vivo Lentiviral vectors | • Fanconi Anemia (FA)  
• Leukocyte Adhesion Deficiency-I (LAD-I)  
• Pyruvate Kinase Deficiency (PKD)  
• Infantile Malignant Osteopetrosis (IMO) |
| In-vivo AAV | Monogenic Multi-organ Disease |

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

**Near-term Milestones (2018)**  
• Additional clinical data for FA expected over the next 12-18 months  
• Disclosure of AAV program (2H18)  
• Additional programs expected to advance towards the clinic (next 12-18 months)

**Medium-term Milestones (2019-2021)**  
• FA advances to potential registration trial stage (expected in 2019)  
• Registration trials for currently planned programs; first BLA submissions  
• 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 lenti- & AAV-based therapies  
• Clearly-defined product metrics across indications  
• Experienced company leaders  
• Leading research & manufacturing partners
## Pipeline-at-a-Glance

<table>
<thead>
<tr>
<th>Therapies</th>
<th>Discovery</th>
<th>Preclinical</th>
<th>Clinical</th>
<th>Commercial</th>
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<tbody>
<tr>
<td>Fanconi Anemia (FA)</td>
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<td>Leukocyte Adhesion Deficiency-I (LAD-I)</td>
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<td>Undisclosed AAV</td>
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<td>CRISPR/Cas9 for FA</td>
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Leveraging the Full Spectrum of GTx Platforms

**In Vivo**
AAV Gene Therapy

- Laboratory-produced AAV

**Ex Vivo**
Lentiviral Gene Therapy

- Remove cells & isolate patient HSCs
- Laboratory-produced LV

Therapeutic LVV

Gene-modify HSCs

Therapeutic AAV

Direct intravenous injection

Infusion of modified HSCs
Fanconi Anemia (FA)

**Background:**

- **Etiology:** FANC-A gene mutation → impaired DNA repair  
- **Pathology:** Bone marrow failure by age 10. Increased cancer risk of 30-50 fold (Acute Myeloid Leukemia and Head and Neck most common) \(^1\)  
- **Current available treatment:** HSCT, associated w/ GVHD  
- **Prevalence:** ~2,000 in US/EU  
  - ~75-80 transplants/yr in US/EU \(^2\)  
  - ~30-40% of pts receive transplant \(^3\)  
- **RP-L102 potential market est.:** >250 patients/year  

**Upcoming Milestones:**

- Additional clinical data over the next 12-18 months  
- Advance to global registration trial stage in 2019

---

\(^1\) Alter Br J Haematol 2010; \(^2\) CIBMTR and EBMT registries 2009-2013; \(^3\) Alter BP et al. Haematologica 2017
Rationale for Gene Therapy in FA: Somatic Mosaicism = “Natural” GTx

Somatic mosaicism in FA leads to stabilization/correction of blood counts, in some cases for decades. This uncommon variant results from a reverse mutation and 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.  

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Gene Therapy Value Proposition: Early, Low-toxicity Intervention to Prevent Hematologic Failure

Gene therapy in FA:

- Potential to correct blood & bone marrow defect without conditioning
- No/limited hospitalization or transplant-unit medical care required
- No anticipated further increase in risk of head and neck cancer
- GTx implemented as preventative measure to avert bone marrow failure; BMT is indicated for patients in whom marrow failure has occurred.
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

**Presidential Symposium:**

Engraftment and Phenotypic Correction of Hematopoietic Stem Cells in Non-Conditioned Fanconi Anemia Patients Treated with Ex Vivo Gene Therapy

Dr. Juan Bueren
Results from Phase 1/2 Gene Therapy Trial of RP-L102 in Patients with Fanconi Anemia

Results:

✓ Genetic correction of bone marrow cells (engraftment): Post-treatment peripheral blood VCN increases in all patients. Patient 02002 (first patient with higher RP-L102 dose)
  - 17% at 12 months
  - 34% at 19 months
  - 44% at 24 months

✓ Transduction-enhanced RP-L102 confers marked improvements—Pt 01003 demonstrates highest transduction efficiency and earliest engraftment to date:
  - Manufacturing Improvements: Preliminary drug product VCN of ~2.5-3, more than five-fold higher than the best previously achieved (0.53 for patient 02006 and 0.43 for patient 02002)
  - Genetic Correction of BM cells: Early engraftment was accelerated more than three-fold compared to best previous patients

✓ Durable improvements consistent with somatic mosaicism:
  - Phenotypic correction of blood cells (DEB Assay): improvement in chromosomal stability of T-lymphocytes sustained over several months
  - Phenotypic correction of bone marrow cells (MMC Assay): earlier evidence of gene correction in patients with highest doses (02002 and 02006). In patient 02002, bone marrow resistance to MMC approaches that of healthy donor:
    - 20% at 12 months
    - 70% at 24 months
First Demonstration of Engraftment Without Conditioning (in contrast to Beta-thal, SCD, etc)

Bone Marrow Engraftment: Increasing Levels Confirm Survival Advantage of Gene-Corrected FA Cells

Ciemat Data Presented at ASGCT May 2018
Functional Correction of Bone Marrow

**Progressive Phenotypic Correction of BM Cells (MMC-Resistance)**

MMC assay identifies cells resistant to Mitomycin-C (MMC), a standard DNA damaging agent.

Ciemat Data Presented at ASGCT May 2018
Gene Correction of Bone Marrow Stem Cells: Observed Across Multiple Cell Lineages

Peripheral Blood

Bone Marrow

Ciemat Data Presented at ASGCT May 2018
Safety: Lentiviral Integration Profile Demonstrates Preferential Integration Away from Potentially Oncogenic Transcription Start Sites

Ciemat Data Presented at ASGCT May 2018
Gene Therapy Confers a Phenotype Similar to Somatic Mosaicism

**Improvement of Chromosomal Stability in Presence of DEB**

DEB chromosomal assay measures Diepoxybutane (DEB)-induced chromosome breakage which is elevated in FA.

Ciemat Data Presented at ASGCT May 2018
Increases of Corrected vs. Non-Corrected Leukocytes Support Potential of Gene Therapy to Restore Normal Bone Marrow Function

**Kinetics of Corrected and Uncorrected PB Leukocytes Prior to and After Gene Therapy**

FA-02002  |  FA-02004  |  FA-02005  |  FA-02006

- Uncorrected leukocytes/µL
- Corrected leukocytes/µL

Ciemat Data Presented at ASGCT May 2018
Gene Therapy Stabilizes Markedly Declining Blood Counts

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

02004 (Cryo)
(6.9x10^3 cCFU/Kg)
(1.7x10^5 cCD34+/Kg)

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

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

Ciemat Data Presented at ASGCT May 2018
Preliminary Results: Improved Early Engraftment with Transduction Enhancers

First Patient Treated with Transduction-Enhanced RP-L102 (Pt 01003) Shows Early Engraftment Was Accelerated More than Three-fold Compared to the Best Previous Patients

Ciemat Data Presented at ASGCT May 2018
FA: Clinical Summary & Path Forward

Current
- FA is a unique bone marrow disease: Somatic Mosaicism leads to “natural gene therapy” and provides compelling rationale for RP-L102 without conditioning.
- Early clinical results, even with a pre-optimized process, suggest RP-L102 can be a transformative therapy.

Upcoming
- Additional patients with optimized process, including transduction enhancers, may demonstrate faster engraftment.
- If needed, antibody-based non-genotoxic conditioning approaches as a backup future development strategy.

Regulatory
- Goal is to achieve accelerated approval and incorporate GTx for FA early in life as preventative for BM failure.
Leukocyte Adhesion Deficiency-I (LAD-I)

**Background:**
- ITGB2 gene mutation → impaired CD18 expression & WBC migration → severe infections
- ~50% patients w/severe variant → ~2/3 mortality by age 2
- Current available treatment: HSCT, associated with GVHD
- GTx potential market est. >25-50 patients/year

**Upcoming Milestones:**
- Target IMPD filing in Spain in 4Q18
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.


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).

LAD-I: Improved Process Produces VCN >2.4

VCN in Liquid Culture

Utilizing GMP vector branch

Source: Company data on file
# LAD-I Program Summary

## Ultra-rare Disease = Streamlined Regulatory Approach

| Potential design & endpoints for approval | Target Patient Population:  
Severe LAD-I patients (CD18<2%), ~2/3 mortality by 2y |
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<tbody>
<tr>
<td></td>
<td><strong>Control:</strong> Lit review of ~300 pts. (Rocket published*)</td>
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<tr>
<td></td>
<td><strong>Potential approval path:</strong> Early Endpoint Read: Modest correction of CD18 expression</td>
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## Efficacy Trials & Registration Status

| Registration & study planning on-schedule | • 3 global sites planned in the US/EU  
• Recruitment underway from around the globe  
• US PI identified  
• IMPD submission planned in 4Q2018 (Spain)  
• PoC data expected in 2019 |

## Product/Manufacturing Optimization

| Advancing toward optimization | • GMP vector production ongoing  
• Vector manufacturing/transduction optimization underway  
  o VCN approx. 2-4 with latest batch (Target VCN>1)  
  o Tdx enhancers to further enhance VCN |

---

Pyruvate Kinase Deficiency (PKD)

Background:
- PKLR gene mutation → shortage of RBC ATP → hemolytic anemia
- Current available treatment: transfusions, splenectomy
- GTx potential market est. >250 patients/year

Upcoming Milestones:
- Rolling IMPD filing planned in early 2019
### Product/Manufacturing Optimization

**Positive outlook for near term optimization PoC**
- Expected effective engraftment requirement < 50%
- Optimization of vector manufacturing + transduction process in progress
- VCN now 2-4 range with TDx Enhancers
- GMP vector production slated to begin 2018

### Clinical Efficacy/Registration Status

**Registration & study planning on-schedule**
- Registry efforts underway
- Rolling IMPD submission in the next 12 months
- US site and PI identified
- Plan to treat 2 adults, then 2 pediatric patients in Spain
- 18 post-splenectomy, transfusion-dependent patients pre-identified in EU
Infantile Malignant Osteopetrosis (IMO)

**Background:**
- TCIRG1 gene mutation → dysfunctional osteoclasts
- Bone marrow failure, skeletal deformities, frequent mortality by age 10
- Current available treatment: HSCT
- GTx potential market est. >50 patients/year

**Upcoming Milestones:**
- Clinical trials scheduled to begin in 2019
Background:

• Monogenic multi-organ disease, death in teens without organ Tx
• Vector with on-target MOA, tissue specific tropism
• Current available treatment: Organ Tx
• Prevalence US/EU: 15,000 to 30,000

Upcoming Milestones:

• Preclinical data and disclosure of indication in 2H18
• Target IND filing in next 12 months
AAV: Activity in Mouse Model

Wild-type

KO + AAV.EGFP

KO + AAV.transgene

Undisclosed collaborator data on file
AAV: Expression of Missing Protein in KO mice

Undisclosed AAV GTx/GAPDH Ratio

Undisclosed collaborator data on file
### Growing IP Portfolio

<table>
<thead>
<tr>
<th>4 in-licensed patent families for GTx products and related tech</th>
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<tbody>
<tr>
<td><strong>Supporting current pipeline efforts</strong></td>
</tr>
<tr>
<td>• In-licensed three pending international patent applications filed under Patent Cooperation Treaty (PCT) for FA, PKD &amp; LAD programs</td>
</tr>
<tr>
<td>• One pending PCT application for undisclosed AAV-based GTx</td>
</tr>
<tr>
<td><strong>Efforts underway to protect and enhance proprietary technology</strong></td>
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<tr>
<td><strong>Securing protection for continued growth</strong></td>
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<tr>
<td>• Additional pending patent applications in the US, Europe and Japan relating to devices, methods, and kits for harvesting and genetically modifying target cells</td>
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World-Class Research and Manufacturing Partners

- CIBER
- El CIEMAT
- Fred Hutchinson Cancer Research Center
- IIS FJD
- Lund University
- Memorial Sloan Kettering Cancer Center
- MolMed S.p.A.
- Stanford Medical School
### Leadership Team - Expertise in GTx & Rare Diseases Clinical Development

<table>
<thead>
<tr>
<th>Name</th>
<th>Title</th>
<th>Expertise</th>
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<tbody>
<tr>
<td>Gaurav Shah, M.D.</td>
<td>President &amp; Chief Executive Officer</td>
<td>Led multiple biologics approvals</td>
</tr>
<tr>
<td>Jonathan Schwartz, M.D.</td>
<td>Chief Medical Officer &amp; Head of Clinical Development</td>
<td>Spearheaded Kymriah (CART-19) development at Novartis towards approval</td>
</tr>
<tr>
<td>Kinnari Patel, Pharm.D., MBA</td>
<td>Chief Operating Officer &amp; Head of Development</td>
<td>Led Opdivo and six rare disease indication approvals</td>
</tr>
<tr>
<td>Raj Prabhakar, MBA</td>
<td>SVP, Business Operations &amp; Corporate Strategy</td>
<td>~17 years cell, gene and biotech Business development</td>
</tr>
<tr>
<td>Claudine Prowse, Ph.D.</td>
<td>SVP, Head of Corporate Development &amp; IRO</td>
<td>~20 years capital markets, strategy, corporate development</td>
</tr>
<tr>
<td>Christopher Ballas, Ph.D.</td>
<td>Vice President, Manufacturing</td>
<td>~20 years in cell and gene therapy development &amp; manufacturing</td>
</tr>
<tr>
<td>Gayatri R. Rao, M.D., J.D.</td>
<td>Vice President, Regulatory Policy and Patient Advocacy</td>
<td>7-Year Former Director of FDA’s Office of Orphan Products Development</td>
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- NewYork-Presbyterian Well Cornell Medical Center
- Lilly
- Mount Sinai
- ImClone Systems
- Novartis
- Columbia University
- Bristol-Myers Squibb
- Pfizer
- Novartis
- AstraZeneca
- Roche
- caladrius BIOSCIENCES
- Celsion Corporation
- Osiris Therapeutics, Inc.
- Biogen
- IONIS Pharmaceuticals
- HUMAN GENOME SCIENCES
- LEERINK
- 華新康德
- WuXi AppTec
- FDA U.S. FOOD & DRUG ADMINISTRATION
- SIDLEY

**~** indicates approximate years of experience.
Near-Term Potential Clinical Value Drivers

- 2Q18: FA: Updated Patient Data Presented at ASGCT

- 2H18: AAV: First disclosure of indication
  - LAD-I: Target IMPD filing

- 2019: Up to Four Programs in the Clinic
  - Clinical Data expected in up to Two Programs