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.
Gene Therapy: Why Now?

**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
- Infusion of modified HSCs
- Therapeutic LVV
- Gene-modify HSCs
# About Rocket Pharma

A 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>
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<tr>
<td></td>
<td>Infantile Malignant Osteopetrosis (IMO)</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>In-vivo adeno-associated virus (AAV)</th>
<th>Danon Disease</th>
</tr>
</thead>
</table>

## 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 (Next 12-18 months)
- FA and LAD-I advance to potential registration trial stage

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

- Ongoing registration trials for currently planned programs; first BLA submission
- 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>Experience</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Gaurav Shah, M.D.</strong></td>
<td>President &amp; Chief Executive Officer</td>
<td>~20 years cell and gene therapy expertise</td>
</tr>
<tr>
<td><strong>Jonathan Schwartz, M.D.</strong></td>
<td>CMO &amp; SVP, Clinical Development</td>
<td>~17 years cell, gene and biotech business development</td>
</tr>
<tr>
<td><strong>Kinnari Patel, Pharm.D., MBA</strong></td>
<td>COO &amp; EVP, Development</td>
<td>~20 years cell and gene therapy development &amp; manufacturing</td>
</tr>
<tr>
<td><strong>Annahita Keravala, Ph.D.</strong></td>
<td>AVP, AAV Platform</td>
<td>7-Year Former Director of FDA’s Office of Orphan Products Development</td>
</tr>
<tr>
<td><strong>Gayatri R. Rao, M.D., J.D.</strong></td>
<td>VP, Reg Policy &amp; Patient Advocacy</td>
<td>~17 years cell, gene and biotech business development</td>
</tr>
<tr>
<td><strong>Raj Prabhakar, MBA</strong></td>
<td>SVP, Bus Operations &amp; Bus Development</td>
<td>~20 years capital markets, strategy, corporate development</td>
</tr>
<tr>
<td><strong>Claudine Prowse, Ph.D.</strong></td>
<td>SVP, Strategy &amp; Corporate Dev</td>
<td>~20 years cell and gene therapy development &amp; manufacturing</td>
</tr>
<tr>
<td><strong>Christopher Ballas, Ph.D.</strong></td>
<td>VP, Manufacturing</td>
<td>15+ years cell and gene therapies expertise</td>
</tr>
<tr>
<td><strong>Brian C. Beard, Ph.D.</strong></td>
<td>AVP, CMC Lenti &amp; AAV</td>
<td></td>
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</tbody>
</table>
### Rocket’s Expanding Pipeline: Potential for Significant Value Creation Near and Long Term

<table>
<thead>
<tr>
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<tbody>
<tr>
<td>Discovery</td>
<td></td>
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<tr>
<td>Preclinical</td>
<td></td>
<td></td>
<td>Process A in the U.S. &amp; E.U.</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Phase 1</td>
<td></td>
<td></td>
<td>Process B in the E.U.*</td>
<td></td>
<td></td>
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<tr>
<td>Phase 2</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Designations</td>
<td>Fast Track, Orphan Drug (U.S.)</td>
<td>RMAT, ATMP, Fast Track, Rare Pediatric, Orphan Drug (U.S./E.U.)</td>
<td>ATMP, Fast Track, Rare Pediatric, Orphan Drug (U.S./E.U.)</td>
<td>Orphan Drug (U.S./E.U.)</td>
<td>Orphan Drug (U.S.)</td>
</tr>
</tbody>
</table>

*Phase 1/2
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 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
RP-A501 Restores Cardiac Function in KO Mice

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

**Cardiac Contractility**

![Graph showing dose-dependent improvements in cardiac contractility](image)

**Cardiac Relaxation**

![Graph showing dose-dependent improvements in cardiac relaxation](image)

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**

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

*Mouse LAMP2 and Human LAMP2 data are from separate Western blots.*

**LC3-II Protein Expression**

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></td>
<td>Wild Type</td>
<td>KO Control</td>
</tr>
<tr>
<td><strong>Heart</strong></td>
<td><img src="image1" alt="Wild Type" /></td>
<td><img src="image2" alt="KO Control" /></td>
</tr>
<tr>
<td><strong>Liver</strong></td>
<td><img src="image6" alt="Wild Type" /></td>
<td><img src="image7" alt="KO Control" /></td>
</tr>
<tr>
<td><strong>Skeletal Muscle</strong></td>
<td><img src="image11" alt="Wild Type" /></td>
<td><img src="image12" alt="KO Control" /></td>
</tr>
</tbody>
</table>
Dose-dependent Widespread LAMP2 Expression in Cardiac Tissue
• RP-A501 Shows Phenotype Improvements:
  - 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
No Toxicities Observed in Mouse and Monkey Models

- **RP-A501 Preclinical Safety Profile:**
  - 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
RP-A501 Clinical Development Plans

2019
- Phase 1 with clinical GMP AAV9 RP-A501 in patients with Danon disease
- Continue registry & patient education/identification
- Clinical retrospective database in progress

2020
- Phase 2/Registrational Study for BLA/MAA submission seeking Accelerated Approval

Natural History Study/Registry (3 year, ~200 patients)

Phase 1
- Phase 2 / Registrational Study for Accelerated/Conditional Approval

Natural History ClinicalTrials.gov Identifier: NCT03766386
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 evaluated²
- Danon Disease Patients with HCM³
  - 85% of males
  - 30% of females

Dilated Cardiomyopathy (DCM)
- Danon Disease Patients with DCM³
  - 15% of males
  - 50% of females

¹Source: 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). <em>No cases of Pompe or Fabry were detected.</em></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

Overview:

- **Current available treatments**: Hematopoietic stem cell transplants associated with GVHD
- **Addressable Market**: 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

Disease Sequelae:
- Birth Defects
- Skin Discoloration
- Developmental Issues
- **Bone Marrow Failure by Age 10**
  - Acute Myeloid Leukemia
  - Head and Neck Cancer ($↑$ risk 30-50x)

Bone Marrow

- **FANC-A Gene Mutation**: Chromosomal breakage
- Platelets
- RBCs
- WBCs

RP-A501
Danon Disease

RP-L102
Fanconi Anemia

RP-L201
Leukocyte Adhesion Deficiency-I

RP-L301
Pyruvate Kinase Deficiency

RP-L401
Infantile Malignant Osteopetrosis

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)

- U.S. 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
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 Levels Provide Evidence of Potential Survival Advantage of Gene-Corrected FA Cells

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

Ciemat Data Presented at ASH December 2018

cCFU = Corrected Colony Forming Units; pVCN: Product VCN *Minimally Acceptable Dose
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
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 ASH December 2018
Gene Therapy Stabilizes Markedly Declining Blood Counts. Most Encouraging Counts Where BM Engraftment is High (>50%)*

Ciemat Data Presented at ASH December 2018

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

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

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

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

BM = Bone Marrow; cCD34+ = Corrected CD34+ cells; cCFU = Corrected Colony Forming Units
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: [ghr.nlm.nih.gov/condition/fanconi-anemia#statistics](https://ghr.nlm.nih.gov/condition/fanconi-anemia#statistics)
\(^3\)Source: [https://www.stjude.org/disease/fanconi-anemia.html](https://www.stjude.org/disease/fanconi-anemia.html)
Overview:

• **Background:** Disorder characterized by recurring and potentially fatal infections caused by ITGB2 gene mutations
  - ~75% patients with severe variant: ~2/3 mortality by age 2

• **Current available treatments:** Hematopoietic stem cell transplants associated with 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 in the US; Advance Therapy Medicinal Product (ATMP) classification in EU
# 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> Lit review of ~300 pts. (Rocket/academic collaborative publication*)</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

| Registration & study planning on-schedule | ✓ Orphan Drug (U.S./E.U.) and Pediatric Rare Disease (U.S.) designations granted |
|                                          | ✓ IND & Phase 1/2 cleared by FDA |
|                                          | ✓ Spain IMPD cleared |
|                                          | ❏ 3 global sites planned in the US/EU |
|                                          | ❏ Recruitment underway from around the globe |
|                                          | ❏ US PI identified |

## 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.

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 batch

Source: Company data on file
Pyruvate Kinase Deficiency (PKD)
Monogenic Red Blood Cell Disorder

Overview:

- Current available treatments: Chronic blood transfusions and splenectomy—side effects include iron overload and hemolysis
- Addressable Market: ~250-500 patients/year
- RP-301: 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

1Market 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.
**PKD Program Summary**

### Product/Manufacturing Optimization

<table>
<thead>
<tr>
<th>Positive outlook for near term optimization PoC</th>
</tr>
</thead>
<tbody>
<tr>
<td>- Target effective engraftment of 30-40%</td>
</tr>
<tr>
<td>- Optimization of vector manufacturing</td>
</tr>
<tr>
<td>+ transduction process</td>
</tr>
<tr>
<td>- VCN now 2-4 range with TDx Enhancers</td>
</tr>
</tbody>
</table>

### Clinical Efficacy/Registration Status

<table>
<thead>
<tr>
<th>Registration &amp; study planning on-schedule</th>
</tr>
</thead>
<tbody>
<tr>
<td>✓ Registry efforts underway</td>
</tr>
<tr>
<td>✓ US site identified as Stanford University</td>
</tr>
<tr>
<td>✓ Plan to treat 2 adults, then 2 older and then 2</td>
</tr>
<tr>
<td>younger pediatric patients</td>
</tr>
<tr>
<td>✓ 18 post-splenectomy, transfusion-dependent</td>
</tr>
<tr>
<td>patients pre-identified in EU</td>
</tr>
</tbody>
</table>
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 with targeted therapies in development\(^2\)

\(^1\)Source: Blood. 2000 Jun 1;95(11)-3585-8.
\(^2\)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

---

\(^1\)Source: [https://www.orpha.net/consor/cgi-bin/OC_Exp.php?lng=EN&Expert=667](https://www.orpha.net/consor/cgi-bin/OC_Exp.php?lng=EN&Expert=667)

\(^2\)Note: Estimated incidence of one in 200,000 live births; Source: [http://www.orpha.net/consor/cgi-bin/OC_Exp.php?lng=EN&Expert=667](http://www.orpha.net/consor/cgi-bin/OC_Exp.php?lng=EN&Expert=667)
Growing IP Portfolio

<table>
<thead>
<tr>
<th>4 in-licensed patent families for GTx products and related tech</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Supporting current pipeline efforts</strong></td>
</tr>
<tr>
<td>- In-licensed four pending international patent applications</td>
</tr>
<tr>
<td>filed under Patent Cooperation Treaty (PCT):</td>
</tr>
<tr>
<td>- FA</td>
</tr>
<tr>
<td>- LAD-I</td>
</tr>
<tr>
<td>- PKD</td>
</tr>
<tr>
<td>- One pending PCT application:</td>
</tr>
<tr>
<td>- Danon (Licensed through UCSD with worldwide rights to</td>
</tr>
<tr>
<td>AAV9 via REGENXBIO collaboration)</td>
</tr>
<tr>
<td>- Undisclosed patent applications:</td>
</tr>
<tr>
<td>- In-licensed OP intellectual property and know-how</td>
</tr>
<tr>
<td>from Lund and Hannover Universities</td>
</tr>
<tr>
<td><strong>Efforts underway to protect and enhance proprietary technology</strong></td>
</tr>
<tr>
<td><strong>Securing protection for continued growth</strong></td>
</tr>
<tr>
<td>- Additional pending patent applications in the US, Europe</td>
</tr>
<tr>
<td>and Japan relating to devices, methods, and kits for</td>
</tr>
<tr>
<td>harvesting and genetically modifying target cells</td>
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</tbody>
</table>
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.
• REGENXBIO
• Stanford Medical School
• University of California, San Diego
Near and Long Term Value Drivers
Potential for Five Gene Therapy Products to be Approved by 2025

1Q19

- Danon (RP-A501): IND Clearance

2Q19

- FA (RP-L102): FPI with Process B (IND Cleared in Nov ’18)
- Danon (RP-A501): FPI (IND Cleared in Jan ’19)
- LAD-I (RP-L201): FPI for Registration-enabling Phase 1/2 Trial (IND Cleared in Nov ’18)
- PKD (RP-L301): IND Submission

2H19

- FA (RP-L102): Data from Patients Treated Under “Process A” & “Process B”
- FA (RP-L102): Regulatory Alignment on Final Endpoints for Registration
- LAD-I (RP-L201): Phase 1 Data