UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): November 26, 2018

Rocket Pharmaceuticals, Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware	**001-36829**	**04-3475813**
(State or Other Jurisdiction of Incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**

430 East 29th Street, Suite 1040
New York, New York 10016
(Address of Principal Executive Offices)

(646) 440-9100
(Registrant's Telephone Number, Including Area Code)

Check the appropriate box below if the Form 8-K is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instructions A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☒

Item 7.01 Regulation FD Disclosure.

On November 26, 2018, Rocket Pharmaceuticals, Inc. made available an updated corporate presentation on its website. A copy of the corporate presentation is furnished herewith as Exhibit 99.1 and incorporated by reference herein.

In accordance with General Instruction B.2 of Form 8-K, the information in this Current Report on Form 8-K, including Exhibit 99.1, shall not be deemed to be "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, and shall not be incorporated by reference into any registration statement or other document filed under the Securities Act of 1933, as amended, or the Exchange Act, except as shall be expressly set forth by specific reference in such filing.

Item 9.01 Financial Statements and Exhibits

(d) Exhibits

Exhibit No.	Description
99.1	Rocket Pharmaceuticals, Inc. Presentation, dated November 26, 2018

SIGNATURE

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Rocket Pharmaceuticals, Inc.

Date: November 26, 2018

By: /s/ Gaurav Shah
Name: Gaurav Shah
Title: President and Chief Executive Officer

Exhibit 99.1

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.

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	• 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 (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 leaders • Leading research & manufacturing partners

Leadership Team - Expertise in GTx & Rare Diseases Clinical Development

rocket pharma

Gaurav Shah, M.D.
President & Chief Executive Officer

HARVARD UNIVERSITY
NOVARTIS
Lilly
COLUMBIA UNIVERSITY IN THE CITY OF NEW YORK

Spearheaded Kymriah (CART-19) development at Novartis towards approval

Jonathan Schwartz, M.D.
Chief Medical Officer & Head of Clinical Development

NewYork-Presbyterian Weill Cornell Medical Center
Lilly
Mount Sinai
ImClone Systems

Led multiple biologics approvals

Kinnari Patel, Pharm.D., MBA
Chief Operating Officer & Head of Development

Bristol-Myers Squibb
Pfizer
NOVARTIS
Roche
AstraZeneca

Led Opdivo and six rare disease indication approvals

Raj Prabhakar, MBA
SVP, Business Operations & Business Development

caladrius BIOSCIENCES
CPCT
Celsion Corporation
Osiris

~17 years cell, gene and biotech Business development

Claudine Prowse, Ph.D.
SVP, Corporate Strategy & IRO

Biogen
HUMAN GENOME SCIENCES
IONIS
INOTEK
LEERINK

~20 years capital markets, strategy, corporate development

Christopher Ballas, Ph.D.
Vice President, Manufacturing

药明康德 WuXi AppTec
COOK RESEARCH
INDIANA UNIVERSITY SCHOOL OF MEDICINE

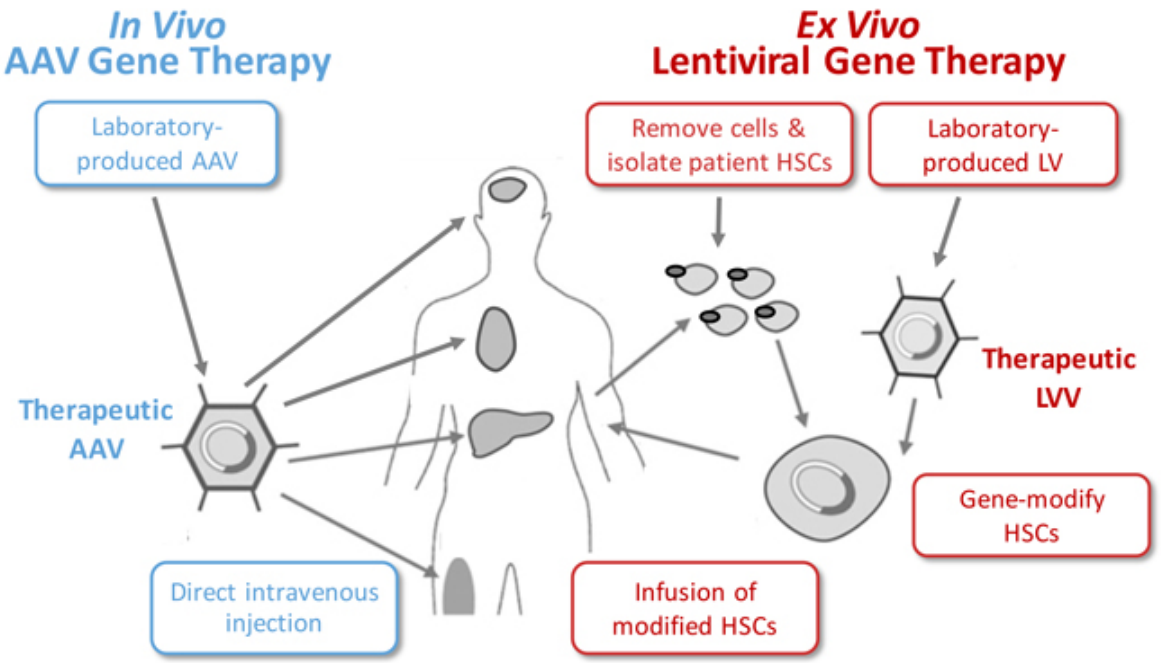
~20 years in cell and gene therapy development & manufacturing

Gayatri R. Rao, M.D., J.D.
Vice President, Regulatory Policy & Patient Advocacy

FDA U.S. FOOD & DRUG ADMINISTRATION
SIDLEY

7-Year Former Director of FDA's Office of Orphan Products Development

Rocket's Expanding Pipeline: Potential for Significant Value Creation Near and Long Term


In Vivo
AAV Gene Therapy
Laboratory-produced AAV
Therapeutic AAV
Direct intravenous injection
Ex Vivo
Lentiviral Gene Therapy
Remove cells & isolate patient HSCs
Laboratory-produced LV
Therapeutic LVV
Gene-modify HSCs
Infusion of modified HSCs

Danon Disease
Monogenic Heart Failure Syndrome

FA

LAD-I

PKD

IMO

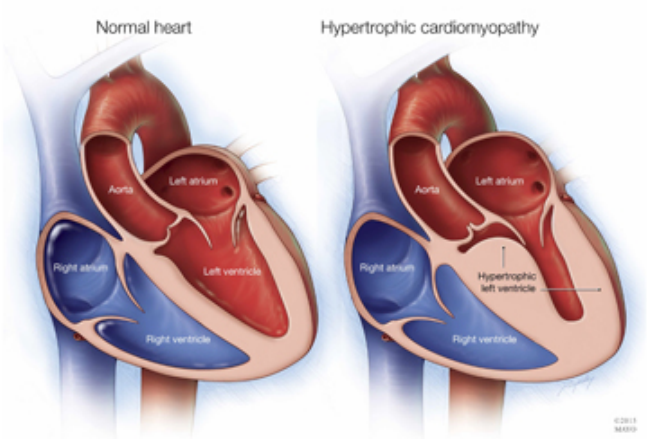
DANON

Background:

- Disease Target
  - Monogenic multi-organ disorder with early mortality primarily due to heart failure
  - Highly penetrant and X-linked dominant
  - No effective therapies
- RP-A501: Rocket's First-In-Class Investigational Gene Therapy
  - Improvements in survival rate and correction of molecular, structural, and phenotypic hallmarks of the disease observed in preclinical studies
  - No toxicities observed in mice and monkeys
  - Strong IP; exclusive and broad rights with REGENXBIO and UCSD
  - IND studies to commence in 1H2019
  - Largest market opportunity of all Rocket programs ~15K-30K patients in US+EU

Most Patients Present with Hypertrophic Cardiomyopathy (HCM)

- Unexplained left ventricular wall thickness and electrophysiological abnormalities
- Disease onset during childhood and adolescence followed by rapid progression to end-stage heart failure and death
- *LAMP2* mutation recently identified in patients with HCM



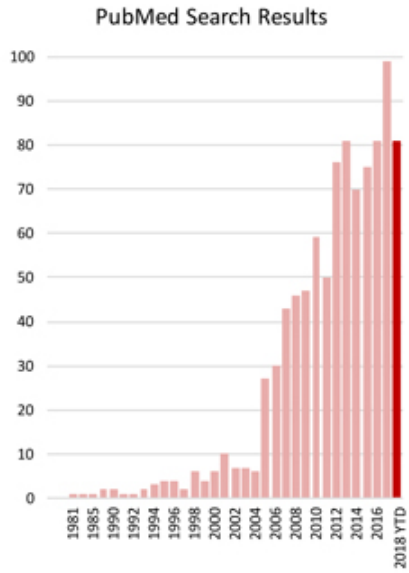
Danon Disease: Newly Discovered with Growing Attention

Recent Clinical and Scientific Progress Has Increased Disease Understanding…

- **1981**: Danon Disease first described[1]
- **2000**: *LAMP2* mutation identified[2]
- **2011-2013**: *LAMP2* inclusion in HCM commercial gene panels[3]
- **2018**: Over 75 unique *LAMP2* mutations identified in literature[4]

…But Danon Disease Is Still Underdiagnosed and Poorly Recognized

- Nonspecific clinical presentation
- Infrequent genetic testing of HCM patients
  - Expensive and not broadly reimbursed
  - No therapeutic reasons for testing
  - Relatively new
- Cardiologist unfamiliarity with disorders of autophagy
- Inaccurate description of *LAMP2* mutation sequelae in early publications



[1]Source: Neurology. 1981 Jan;31(1):51-7.
[2]Source: Nature. 2000 Aug 24;406(6798):906-10.
[3]Sources: JAMA Cardiol. 2018;3(6):520-525. Supplement. Genet Med. 2015 Nov;17(11):880-8.
[4]Sources: Circ Heart Fail. 2014 Sep;7(5):843-9. Neuropathology. 2016 Dec;36(6):561-565. Am J Cardiol. 2016 Sep 15;118(6):888-894. Can J Cardiol. 2016 Nov;32(11):1355.e23-1355.e30. Eur J Med Genet. 2018 May 23. pii: S1769-7212(18)30078-8. Cureus. 2018 Feb; 10(2): e2155.

Danon Disease Prevalence: ~15-30K in the US+EU

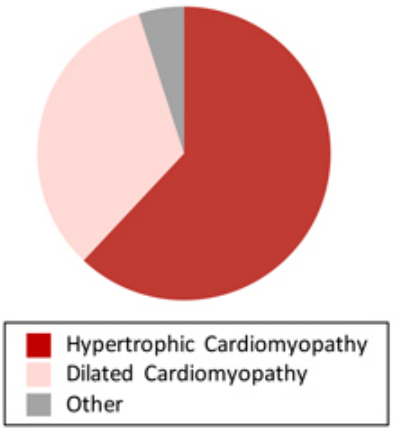
Hypertrophic Cardiomyopathy (HCM)

- 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[1]
  - 85% of males
  - 30% of females

Dilated Cardiomyopathy (DCM)

- Danon Disease Patients with DCM
  - 15% of males
  - 50% of females

US+EU Prevalence:
~15-30,000

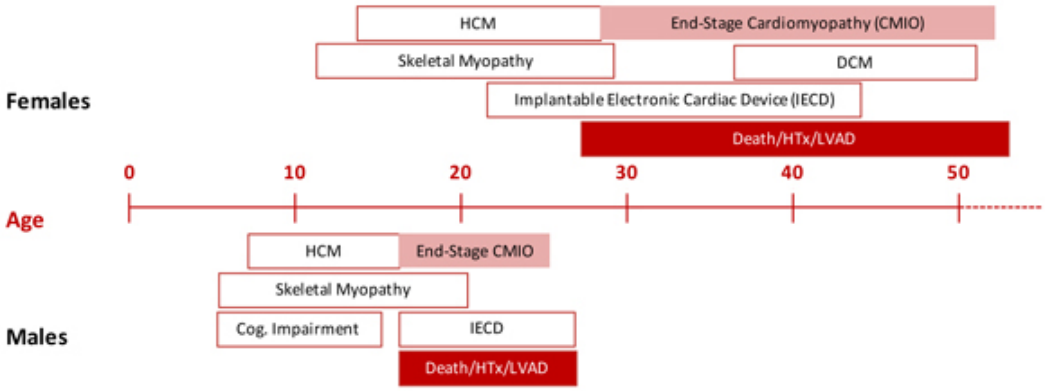


[1]J Am Coll Cardiol. 2015 Mar 31;65(12):1249-1254.
[2]Heart 2004;90:842–846. N Engl J Med. 2005 Jan 27;352(4):362-72. Genet Med. 2015 Nov;17(11):880-8. Cardiovasc J Afr. 2016 May-Jun; 27(3): 152–158. J. of Cardiovasc. Trans. Res. (2017) 10:35–46.

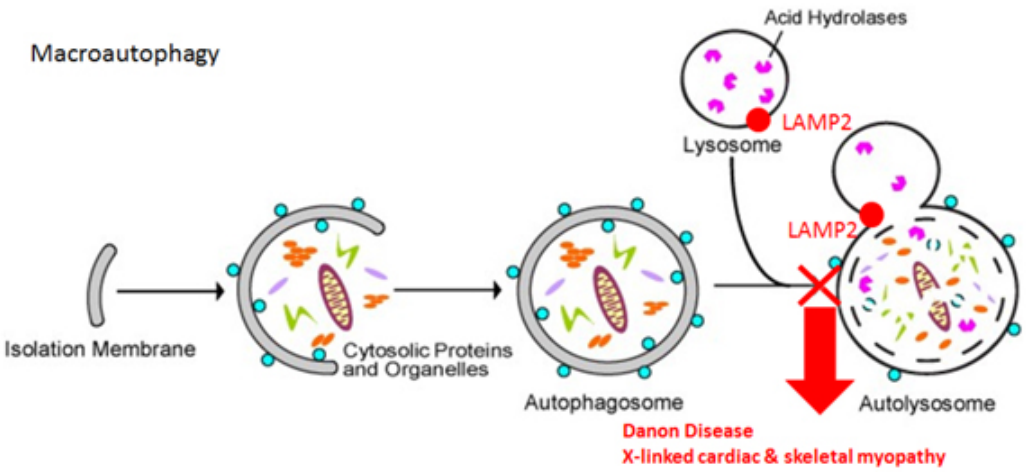
Danon Disease: Devastating Multisystemic Disorder with No Specific Treatments

- **95% of patients have severe cardiomyopathy**
  - Patients die from progressive heart failure
  - Males frequently die in their teens and females die in their thirties and forties
- **Other clinical manifestations**
  - Skeletal Myopathy
  - CNS manifestations
  - Liver disease manifests as elevations of liver enzymes
- **Heart transplant is not curative and is associated with considerable morbidity and mortality**

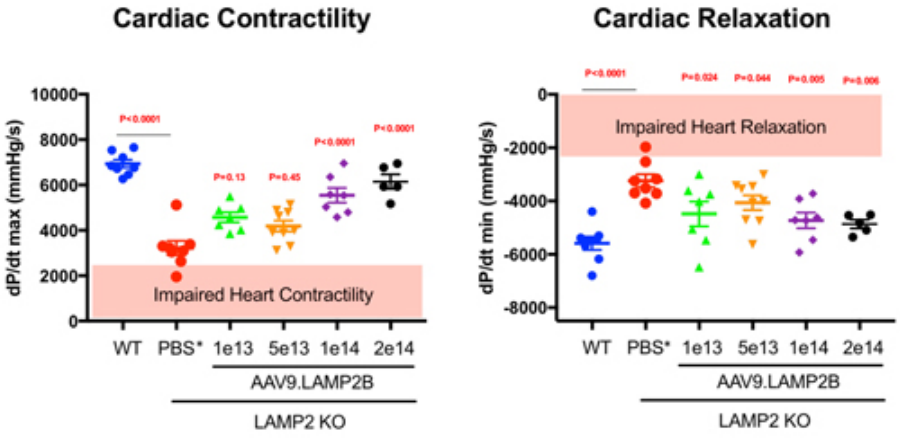
Danon Disease Clinical Presentation and Intervention Timeline



Danon Disease: An Impairment in Autophagy Caused by *LAMP2B* Mutations

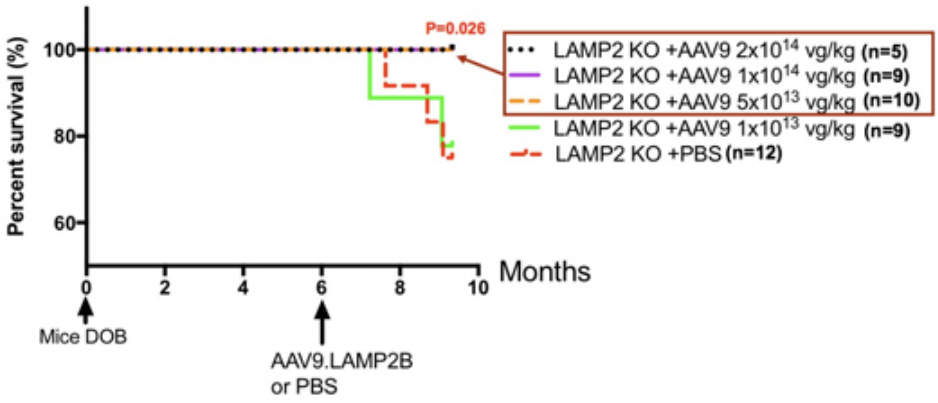


Dose-Dependent Improvements in Systolic and Diastolic Function in LAMP2 KO Mice



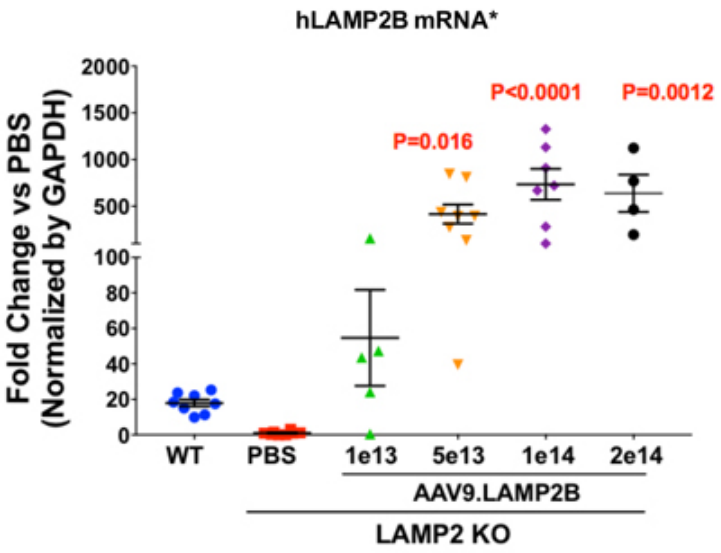
*PBS = Phosphate Buffered Saline (Negative Control)

RP-A501 Shows Survival Benefit at Higher Doses



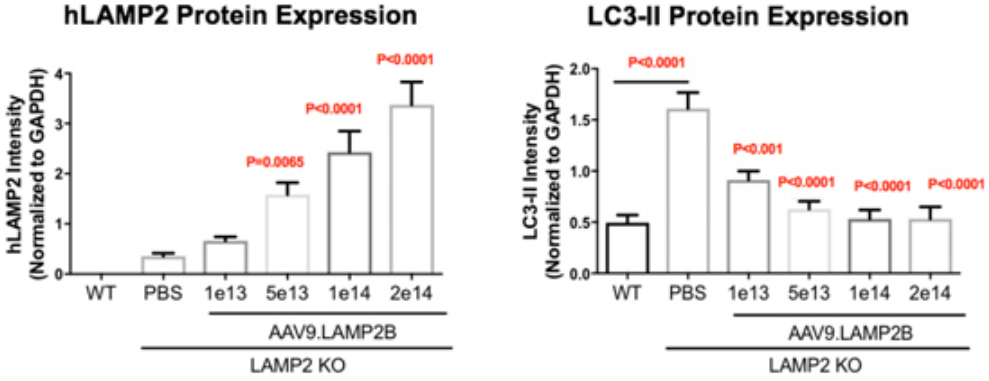
Note: All mice were sacrificed at Month 10

RNA: RP-A501 Elicits Expression of hLAMP2B mRNA in Cardiac Tissue of KO Mice

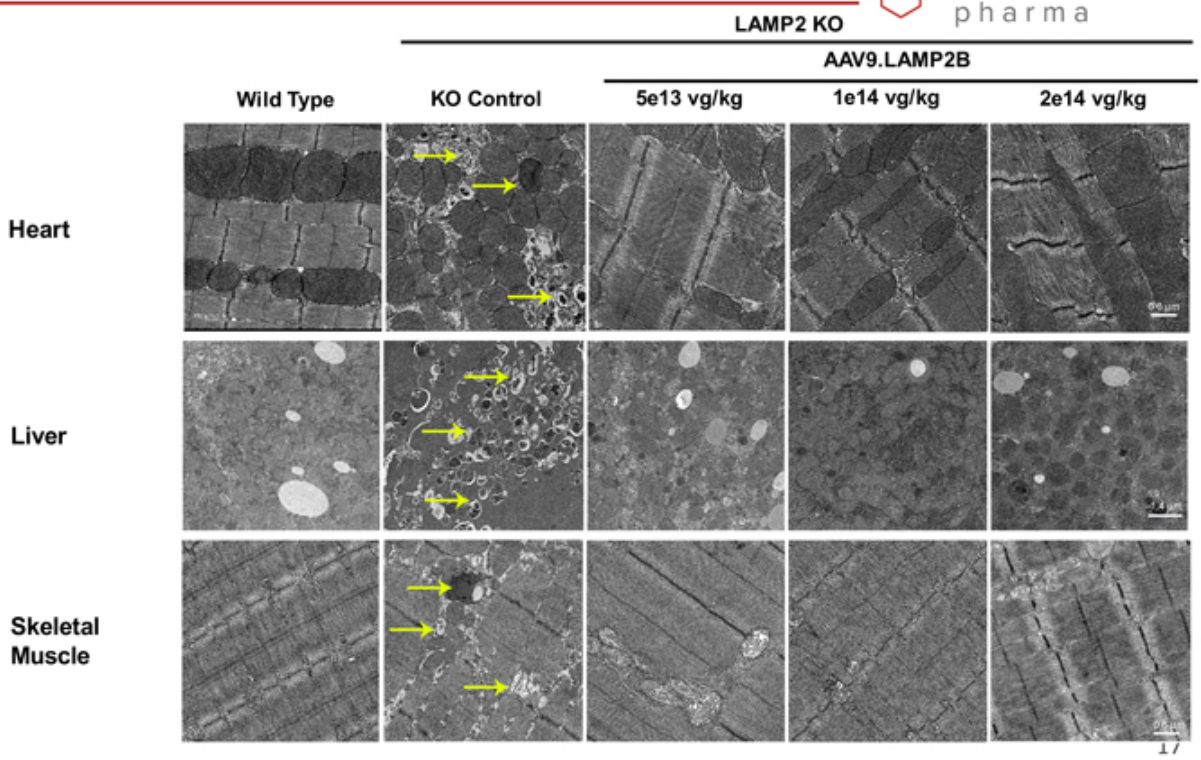


*hLAMP2B = Human LAMP2B

Protein: RP-A501 Elicits Expression of LAMP2B Protein and Autophagic Flux in Heart



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



- **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

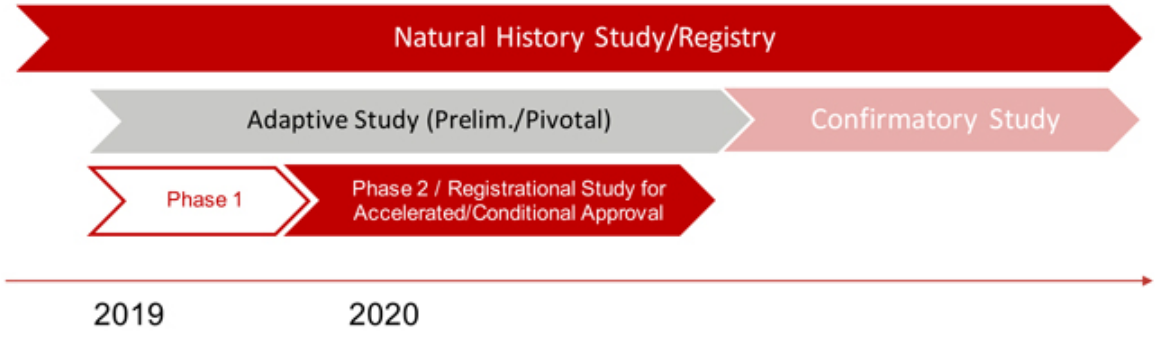
RP-A501 Clinical Development Plans

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



Fanconi Anemia (FA)
Monogenic DNA-repair disorder

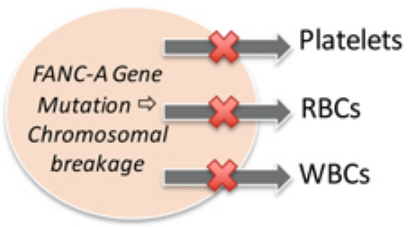
FA

LAD-I

PKD

IMO

DANON



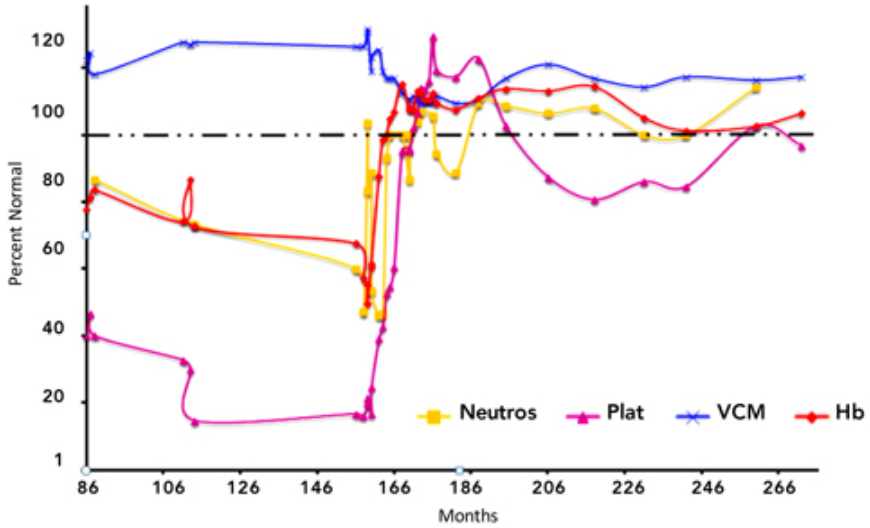
Birth Defects
Skin Discoloration
Developmental Issues
Bone Marrow Failure by Age 10
Acute Myeloid Leukemia
Head and Neck Cancer[1]
(↑ risk 30-50x)

- **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.** : 400-500 patients/year (Non-conditioned patients)

[1] Alter Br J Hametol 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

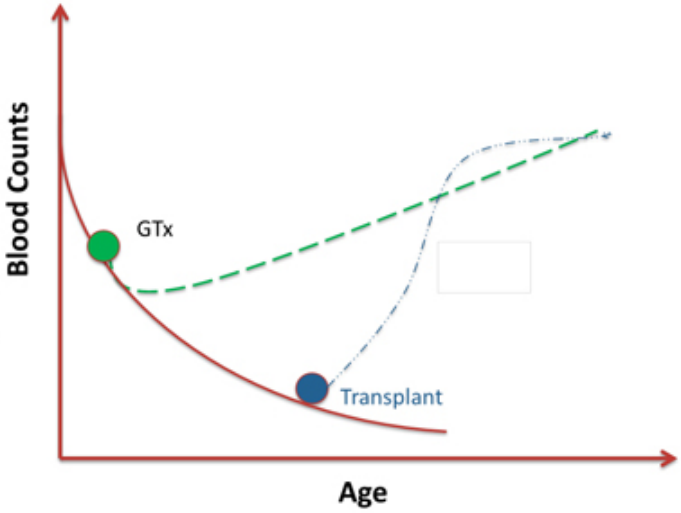
Somatic mosaicism in FA leads to stabilization/correction of blood counts, in some cases for decades. This uncommon variant results from a reverse mutation and demonstrates that a modest number of gene-corrected hematopoietic stem cells can repopulate a patient's blood and bone marrow with corrected (non-FA) cells. [1,2]



[1] Soulier, J., et al. (2005) Detection of somatic mosaicism and classification of Fanconi anemia patients by analysis of the FA/BRCA pathway. *Blood* 105: 1329-1336; [2]Data on file: Showing a single patient with a spontaneous correction of blood counts, no therapy administered

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.



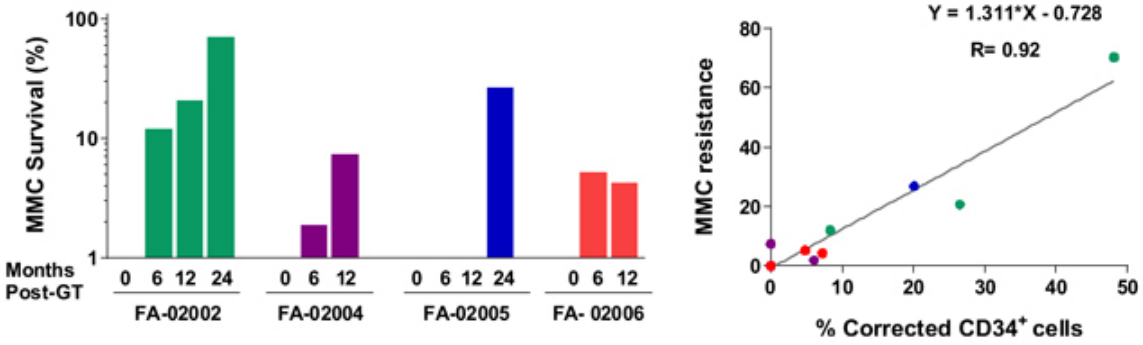
Key efficacy measurements:

- **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

21st Annual Meeting
ASGCT
CHICAGO, ILLINOIS
2018
MAY 16-19

EUROPEAN SOCIETY OF GENE & CELL THERAPY

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 Therapy Confers a Phenotype Similar to Somatic Mosaicism

Improvement of Chromosomal Stability in Presence of DEB

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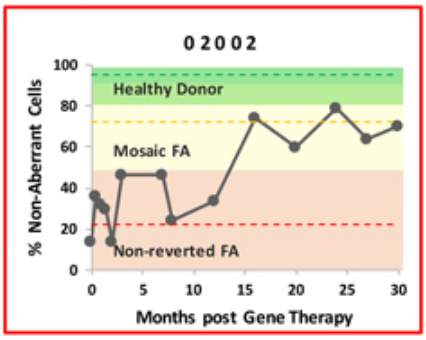

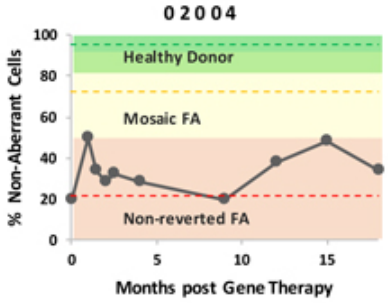

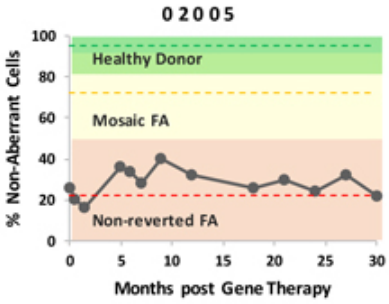

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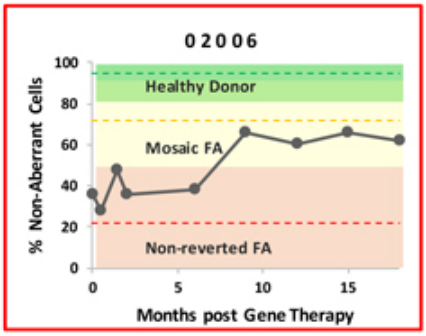

Ciemat Data Presented at ESGCT Oct 2018

DEB chromosomal assay measures Diepoxybutane (DEB)- induced chromosome breakage which is elevated in FA *Minimally Acceptable Dose

Bone Marrow Engraftment: Increasing Levels Provide Evidence of Potential Survival Advantage of Gene-Corrected FA Cells

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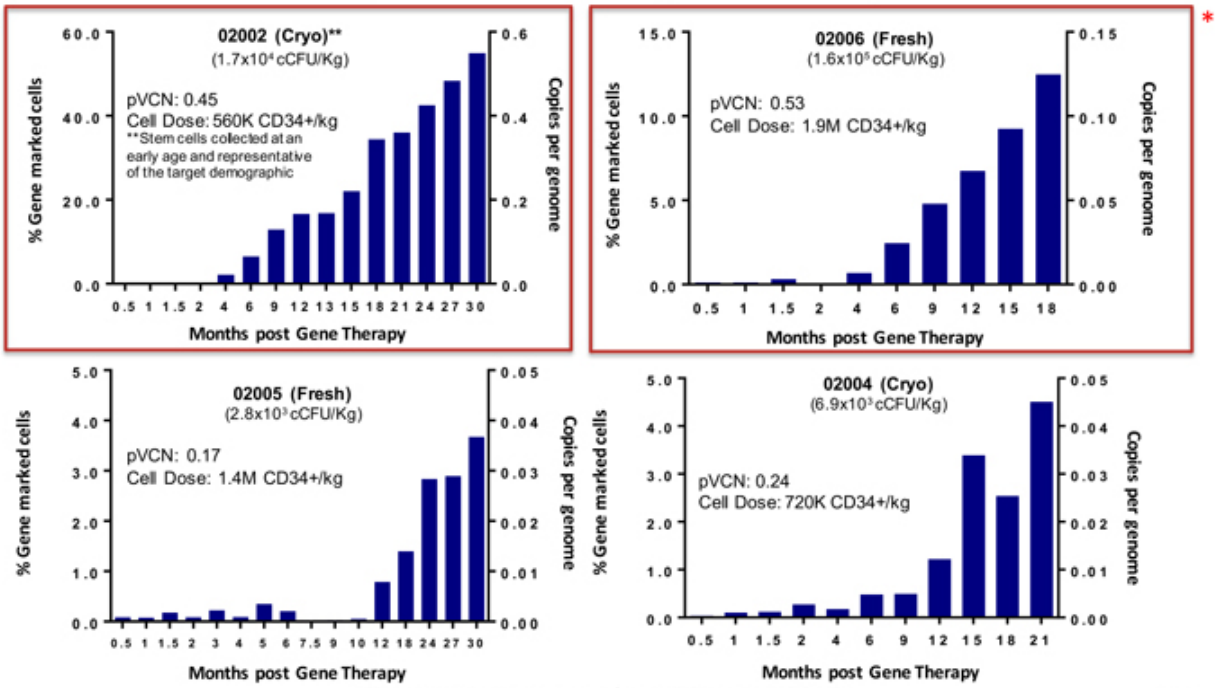
First Demonstration of Engraftment Without Conditioning ("Process A"—non-optimized—RP-L102)



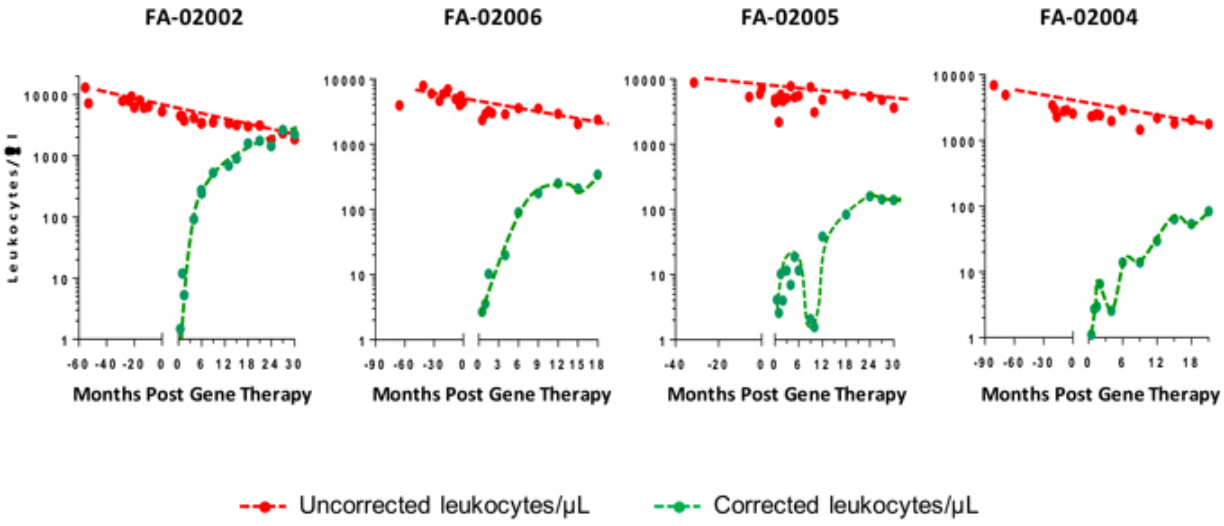
Ciemat Data Presented at ESGCT Oct 2018

cCFU = Corrected Colony Forming Units *Minimally Acceptable Dose

Increases of Corrected Leukocytes Support Restoration of Normal Bone Marrow Function Consistent with Mosaic Phenotype

rocket pharma

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



Ciemat Data Presented at ESGCT Oct 2018

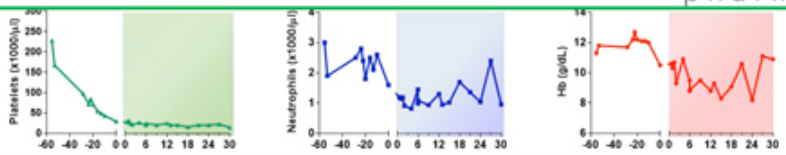
Gene Therapy Stabilizes Markedly Declining Blood Counts

*Most Encouraging Counts Where Engraftment is High (>50%)**

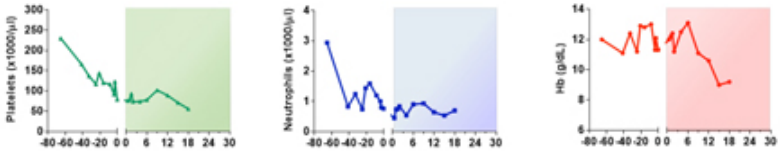
rocket pharma

*

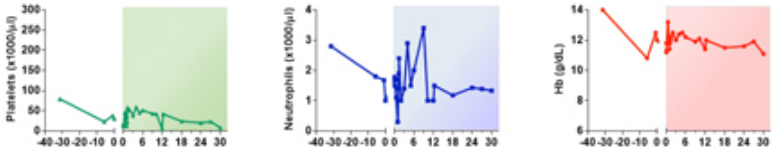
02002 (Cryo)
2.5x10^5 cCD34$^+$/Kg
1.7x10^4 cCFU/Kg



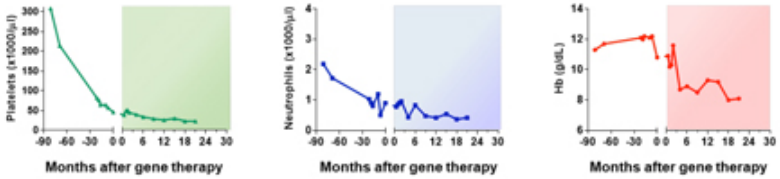
02006 (Fresh)
4.0x10^5 cCD34$^+$/Kg
1.6x10^5 cCFU/Kg



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



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



Ciemat Data Presented at ESGCT Oct 2018

cCD34+ = Corrected CD34+ cells cCFU = Corrected Colony Forming Units

FA: Clinical Summary & Path Forward

Process A (non-optimized)

- Interim data from patients under **"Process A"** (12-month follow up) showed:
  - Durable engraftment for up to 30 months
  - Continued improvement in phenotypic markers MMC and DEB
  - Continued stabilization of previously-declining blood count
  - Progressive increases of corrected versus non-corrected peripheral blood cells"
- "Process A" patients will continue to be followed

Process B (higher cell doses, transduction enhancers, and commercial-grade vector)

- U.S. clinical trial expected to begin in 2019 (~12 patients) with sites at the Center for Definitive and Curative Medicine at Stanford University School of Medicine, Hospital Niño Jesús/CIEMAT, and other leading centers in the U.S. and in the EU.
- No conditioning is required
- Expect to finalize registrational plans in 2H19

Leukocyte Adhesion Deficiency-I (LAD-I)

Monogenic immunodeficiency disease

FA

LAD-I

PKD

IMO

DANON

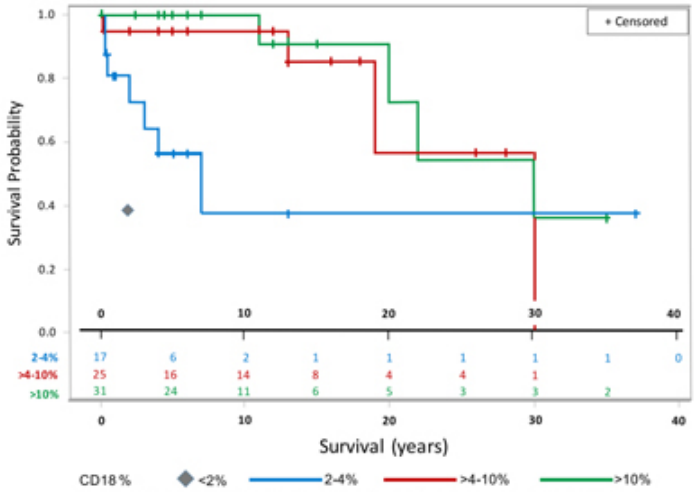
Background:

- **ITGB2 gene mutation** (encodes the CD18 protein) → leads to impaired CD18 expression & WBC migration → severe and recurring infections that could be fatal
- **~75%** patients w/severe variant → **~2/3 mortality by age 2**
- **Current available treatment** includes HSCT, but associated w/ GVHD
- **GTx potential market** est.: >25-50 patients/year

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

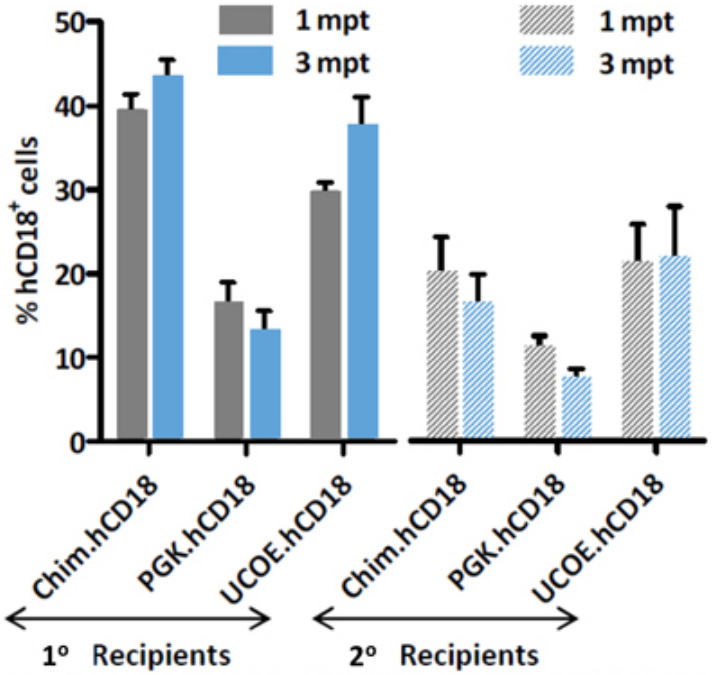
Source: Almarza Novoa E et al. J Allergy Clin Immunol Pract. 2018 Jan 20. pii: S2213-2198(17)31026-7. [Epub ahead of print]

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

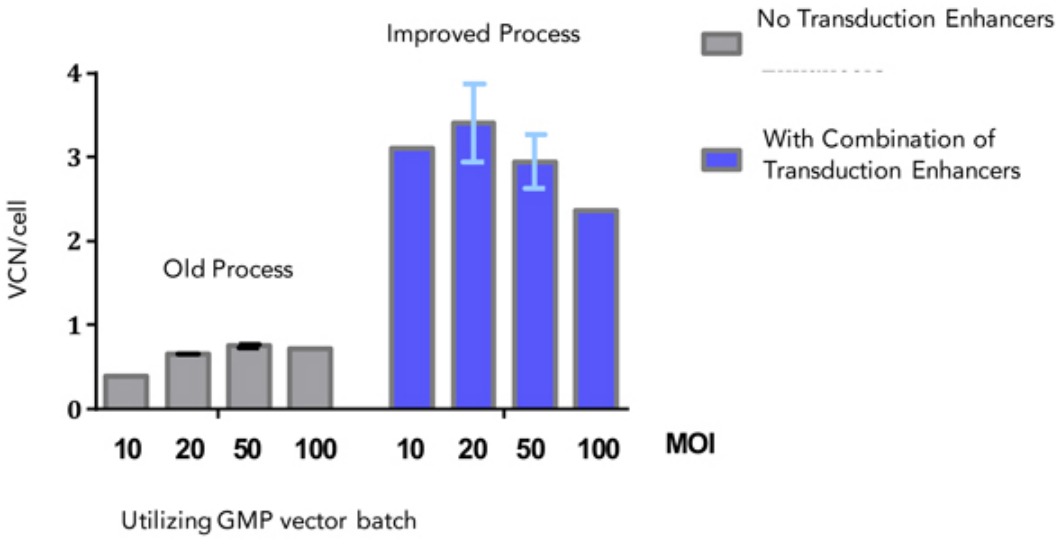
Rocket chose the Chimeric cFES/CTSG (myeloid-specific) promoter (Post-transplant PB VCN 0.4-0.9)



Leon-Rico D, Aldea M, Sanchez-Baltasar R, Mesa-Nuñez C, Record J, Burns SO, Santilli G, Thrasher AJ, Bueren JA, Almarza E. Hum Gene Ther. 2016 Sep;27(9):668-78. doi: 10.1089/hum.2016.016. Epub 2016 May 5.

LAD-I: Improved Process Produces VCN >~2-4

VCN in Liquid Culture



Utilizing GMP vector batch

Source: Company data on file

LAD-I Program Summary

Ultra-rare Disease = Streamlined Regulatory Approach	
Potential design & clinical endpoints	**Target Patient Population:** Severe LAD-I patients (CD18<2%), ~2/3 mortality by 2y **Control:** Lit review of ~300 pts. (Rocket published*) **Potential Clinical Endpoints:** Modest correction of CD18 expression, Survival
Efficacy Trials & Registration Status – Ahead of Schedule	
Registration & study planning on-schedule	✓ IND cleared in November 2018 ❑ 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)

*Almarza Novoa E, Kasbekar S, Thrasher AJ, Kohn DB, Sevilla J, Nguyen T, Schwartz JD, Bueren JA. Leukocyte adhesion deficiency-I: A comprehensive review of all published cases. J Allergy Clin Immunol Pract. 2018 Jan 20. pii: S2213-2198(17)31026-7. doi: 10.1016/j.jaip.2017.12.008.

Pyruvate Kinase Deficiency (PKD)

Monogenic red blood cell disorder

FA

LAD-I

PKD

IMO

DANON

Background:

- Severely affects the pediatric population
- PKLR gene mutation → shortage of RBC ATP → **hemolytic anemia** that can range in severity (mild to severe)
- Current available treatment include **transfusions & splenectomy** which have side effects (iron overload, hemolysis)
- **GTx potential market est.**: ~250-500 patients/year*

*New 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.

PKD Program Summary

Product/Manufacturing Optimization	
Positive outlook for near term optimization PoC	❑ Target effective engraftment requirement < 50% ❑ 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 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)

Monogenic bone resorption disorder

FA

LAD-I

PKD

IMO

DANON

Background:

- Resulting in increased bone density and impaired bone resorption
- TCIRG1 gene mutation → **dysfunctional osteoclasts**
- Bone marrow failure, skeletal deformities, neurologic abnormalities, **frequent mortality by age 10**
- Current available treatment includes **HSCT**, but associated w/ GVHD
- **GTx potential market** est.: >50 patients/year

Growing IP Portfolio

4 in-licensed patent families for GTx products and related tech	
Supporting current pipeline efforts	• In-licensed three pending international patent applications filed under Patent Cooperation Treaty (PCT): ❑ FA ❑ LAD-I ❑ PKD • One pending PCT application: ❑ Danon (Licensed through UCSD with worldwide rights to AAV9 via REGENEXBIO collaboration)
Efforts underway to protect and enhance proprietary technology	
Securing protection for continued growth	❑ 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 Manufacturing Partners

rocket pharma

- CIBER
- El CIEMAT
- Fred Hutchinson Cancer Research Center
- IIS FJD
- Lund University
- Memorial Sloan Kettering Cancer Center
- MolMed S.p.A.
- REGENEXBIO
- Stanford Medical School
- University of California, San Diego

Near-Term Potential Clinical Value Drivers

1H19

- Danon: IND Clearance; FPI
- FA: FPI Phase 1/2 trial (IND cleared in Nov '18)
- FA: Additional Data from Patients Treated under "Process A"
- LAD-I: FPI for registration-enabling Phase 1/2 trial (IND cleared in Nov '18)
- PKD: IMPD/IND submission; FPI

2H19

- FA: data from Patients Treated under "Process B"
- FA: FDA alignment on final endpoints for registration
- LAD-I: Phase 1/2 data
- Four programs in the clinic