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DELTA REPORT

10-K

RCKT - ROCKET PHARMACEUTICALS, I

10-K - DECEMBER 31, 2023 COMPARED TO 10-K - DECEMBER 31, 2022

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TOTAL DELTAS 5331

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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, DC 20549

FORM 10-K

(Mark One)

ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the fiscal year ended **December 31, 2022**
December 31, 2023

OR

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the transition period from to

Commission File Number: 001-36829

Rocket Pharmaceuticals, Inc.

(Exact Name of Registrant as Specified in Its Charter)

Delaware 04-3475813
(State or Other Jurisdiction of
Incorporation or Organization) (IRS Employer
Identification No.)

9 Cedarbrook Drive 08512
, Cranbury, NJ

(Address of Principal Executive Offices) (Zip Code)

(609)
(609) 659-8001

(Registrant's Telephone Number, Including including Area Code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, \$0.01 par value	RCKT	NASDAQ Global Market

Securities registered pursuant to Section 12(g) of the Act: None

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes No

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Exchange Act. Yes

No

Indicate by check mark whether the registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes No

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes No

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company or an emerging growth company. See the definitions of "large" "large accelerated filer," "accelerated" "accelerated filer," "smaller" "smaller reporting company" "company" and "emerging" "emerging growth company" "company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	<input checked="" type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/>	Smaller reporting company	<input type="checkbox"/>
Emerging growth company	<input type="checkbox"/>		<input type="checkbox"/>

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.

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report.

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If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements.

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to §240.10D-1(b).

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes No

The aggregate market value of the common stock held by non-affiliates of the registrant as of ~~June 30, 2022~~ June 30, 2023 (the last business day of the registrant's most recently completed second fiscal quarter) was approximately ~~\$644.3 million~~ \$1.2 billion, based upon the closing price on the NASDAQ Global Market reported for such date.

As of ~~February 22, 2023~~ February 22, 2024, there were ~~79,347,760~~ 90,504,248 shares of common stock, \$0.01 par value per share, outstanding.

Documents Incorporated by Reference

Part III of this annual report on Form 10-K incorporates by reference information (to the extent specific sections are referred to herein) from the registrant's definitive proxy statement for its 2023 2024 Annual Meeting of Stockholders (the "Proxy Statement" "Proxy Statement"). The Proxy Statement will be filed with the United States Securities and Exchange Commission within 120 days of the end of the period covered by this Annual Report on Form 10-K.

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FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K ("Annual Report") contains forward-looking statements that involve risks and uncertainties, as well as assumptions that, if they **never do not** materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this Annual Report are forward-looking statements. In some cases, you can identify forward-looking statements by words such as "aim," "anticipate," "believe," "can," "contemplate," "continue," "could," "design," "develop," "estimate," "expect," "expand," "future," "hope," "intend," "likely," "may," "plan," "potential," "predict," "project," "pursue," "seek," "should," "strategy," "target," "will," "would," or the negative of these words or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- our ability to meet our anticipated milestones for our various drug candidates with respect to the initiation and timing of clinical studies;
 - federal, state, and non-U.S. regulatory requirements, including regulation of our current or any other future product candidates by the U.S. Food and Drug Administration ("FDA");
 - the timing of and our ability to submit regulatory filings, including filings with the FDA, and to obtain and maintain FDA or other regulatory authority approval of, or other action with respect to, our product candidates;
 - our competitors' activities, including decisions as to the timing of competing product launches, pricing and discounting;
 - whether safety and efficacy results of our clinical trials and other required tests for approval of our product candidates provide data to warrant progression of clinical trials, potential regulatory approval or further development of any of our product candidates;
 - our ability to develop, acquire and advance product candidates into, enroll a sufficient number of patients into, and successfully complete, clinical studies, and our ability to apply for and obtain regulatory approval for such product candidates, within currently anticipated timeframes, or at all;
 - our ability to establish key collaborations and vendor relationships for our product candidates and any other future product candidates;
 - our ability to develop our sales and marketing capabilities or enter into agreements with third parties to sell and market any of our product candidates;
 - our ability to **obtain acquire** additional **funding to conduct our planned research businesses, form strategic alliances or create joint ventures and development efforts**;
- Our integration of an acquired business involves a number of risks, including the possibility that the integration process could result in the loss of key employees; the disruption of our ongoing business; or inconsistencies in standards,

controls, procedures, or policies, in each case, that could adversely affect our ability to achieve realize the anticipated benefits benefit of the acquisition, such acquisitions, alliances or joint ventures;

- our ability to successfully develop and commercialize any technology that we may in-license or products we may acquire;
- unanticipated delays due to manufacturing difficulties, including the development of our direct manufacturing capabilities for our AAV programs, and any supply constraints or changes in the regulatory environment; programs;
- our ability to expand our pipeline to target additional indications that are compatible with our gene therapy technologies;
- our ability to successfully operate in non-U.S. jurisdictions in which we currently or in the future do business, including compliance with applicable regulatory requirements and laws;
- uncertainties associated with obtaining our ability to obtain and enforcing enforce patents to protect our product candidates, and our ability to successfully defend ourselves against unforeseen third-party infringement claims;
- anticipated trends and challenges in our business and the markets in which we operate; natural and manmade disasters, including pandemics such as COVID-19, and other force majeures, which could impact our operations, and those of our partners and other participants in the health care industry, and which could adversely impact our clinical studies, preclinical research activities, and drug supply;
- the impact of global economic and political developments on our business, including rising inflation and capital market disruptions, the current conflict in Ukraine, economic sanctions and economic slowdowns or recessions that may result from such developments which could harm our research and development efforts as well as the value of our common stock and our ability to access capital markets;
- our estimates regarding our capital requirements; and
- our ability to obtain additional financing and raise capital as necessary to fund operations or pursue business opportunities.

We caution you that the foregoing list may not contain all of the forward-looking statements made in this Annual Report.

Any forward-looking statements in this Annual Report reflect our current views with respect to future events or to our future financial performance and involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by these forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under Part I, Item 1A. Risk Factors and elsewhere in this Annual Report. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

This Annual Report also contains estimates, projections and other information concerning our industry, our business, and the markets for certain diseases, including data regarding the estimated size of those markets, and the incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties and actual events, or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, we obtained this industry, business, market and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data and similar sources. Unless stated otherwise, references in this Annual Report to "us," "we," "our," or our "Company" and similar terms refer to Rocket Pharmaceuticals, Inc.

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SUMMARY OF THE MATERIAL RISKS ASSOCIATED WITH OUR BUSINESS

Our business is subject to numerous risks and uncertainties that you should be aware of in evaluating our business. These risks and uncertainties include, but are not limited to, the following:

- The outbreak of SARS-CoV-2, which causes COVID-19, has and may continue to adversely impact our business, including our preclinical and clinical studies.
- If, in the future, we are unable to establish sales and marketing capabilities or enter into agreements with third parties to sell and market any of our product candidates, we may not be successful in commercializing those product candidates if and when they are approved.
 - If we fail to obtain necessary additional funding to conduct our planned research and development efforts, we could be forced to delay, reduce, or eliminate our product development programs or commercial development efforts.
 - We have never generated any revenue from product sales and may never be profitable.
 - We may encounter substantial delays in commencement, enrollment or completion of our clinical trials or may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities, which could prevent us from commercializing our current and future product candidates on a timely basis, if at all.
 - If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit, or terminate planned clinical trials, the occurrence of any of which would harm our business, financial condition, results of operations and prospects.
 - Initial Preliminary, interim or interim topline results in our ongoing clinical studies may not be indicative of results obtained when these studies are completed. Furthermore, completed and success in early clinical studies may not be indicative of results obtained in later studies.
 - Our product candidates may cause undesirable and unforeseen side effects or be perceived by the public as unsafe, which could delay or prevent their advancement into clinical trials or regulatory approval, limit the commercial potential or result in significant negative consequences.
 - Our gene therapy product candidates are based on novel technology, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval.
 - Even if we successfully complete the necessary preclinical studies and clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize a product candidate and the approval may be for a narrower indication than we seek.
 - We may never obtain approval for any of our product candidates in the United States ("U.S.") or the European Union ("EU"), or other jurisdictions, which would limit our ability to realize our full market potential.
 - Even if we obtain regulatory approval for a product candidate, we will remain subject to ongoing regulatory obligations and continued regulatory scrutiny.
 - Our If approved, our product candidates may cause undesirable face competition from biosimilars approved through an abbreviated regulatory pathway.
 - Healthcare legislative reform measures may have a material adverse effect on our business and unforeseen side effects or results of operations.
 - If we are successful in commercializing any product, our relationships with customers and third-party payors will be perceived by the public as unsafe, subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could delay expose us to criminal sanctions, civil penalties, exclusion from government healthcare programs, contractual damages, reputational harm and diminished profits and future earnings.
 - We are subject to stringent laws, rules, regulations, policies, industry standards and contractual obligations regarding data privacy and security and may be subject to additional related laws and regulations in jurisdictions into which we expand.
 - We are subject to environmental, health and safety laws and regulations, and we may become exposed to liability and substantial expenses in connection with environmental compliance or prevent their advancement into clinical trials or regulatory approval, limit the commercial potential or result in significant negative consequences. remediation activities.
 - We could experience production problems that result in delays in our development or commercialization programs, limit the supply of our products or otherwise harm our business.
 - We have limited experience in manufacturing, and there can be no assurance that we will be able to successfully manufacture products.
 - Our manufacturing facilities are subject to significant government regulations and approvals, which are often costly and could result in adverse consequences to our business if we fail to comply with the regulations or maintain the approvals.
 - Product liability lawsuits against us could cause us to incur substantial liabilities and could limit the potential commercialization of any products that we may develop.
 - Our ability to successfully develop and commercialize our product candidates will substantially depend upon the availability of reimbursement funds for the costs of the resulting drugs and related treatments.
 - Even if approved, we may not successfully commercialize The commercial success of any of our product candidates, candidates will depend upon the degree of market acceptance of gene therapy by physicians, patients, third-party payors and others in the medical community.

- Ethical, legal, and social issues may reduce demand for any gene therapy products for which we obtain marketing approval.
- We may not be successful in our efforts to expand our pipeline of additional product candidates.
- The success of our research and development activities, clinical testing, and commercialization, upon which we primarily focus, is uncertain.
- We expect to rely on third parties to conduct some or all aspects of our drug product manufacturing, research, and

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preclinical and clinical testing, and these third parties may not perform satisfactorily.

- Disruptions at the FDA and other government agencies caused by funding shortages or global health concerns could hinder their ability to hire, retain or deploy key leadership and other personnel, or otherwise prevent new or modified products from being developed, approved, or commercialized in a timely manner or at all, which could negatively impact our business.
- Our rights to intellectual property for the development and commercialization of our product candidates are subject to the terms and conditions of licenses granted to us by others.
- If we are unable to obtain and maintain patent protection for our products and related technology or are unable to otherwise protect our intellectual property rights and trade secrets related to our product candidates, we may not be able to compete effectively in our markets.
- Changes in U.S. patent law or the patent law of other countries or jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products.
- If we breach our license agreements, it could have a material adverse effect on our commercialization efforts for our product candidates.
- We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property.
- Our business could suffer if it loses the services of, or fails to attract, key personnel.
- We may need to expand our organization and may experience difficulties in managing this growth, which could disrupt our operations.
- Our employees, principal investigators, consultants, and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading which could harm our business.
- Given our commercial relationships outside of the United States, U.S., in particular the European Union, EU, a variety of risks associated with international operations could harm our business.

If we are unable to obtain and maintain adequate patent protection for products and related technology, our ability to successfully commercialize our products may be harmed.

- If we breach our license agreements, it could have a material adverse effect on our commercialization efforts for our product candidates.
- We may be subject to claims challenging the inventorship or ownership of our patents and other intellectual property.
- If we are unable to protect the confidentiality of our trade secrets, our business and competitive position may be harmed.
- If we are unable to obtain or protect intellectual property rights related to our product candidates, we may not be able to compete effectively in our markets.
- We may fail to realize the anticipated benefits of potential acquisitions or business combinations, such as the acquisition combinations.
 - Future formations of Renovacor strategic alliances or joint ventures with third parties could disrupt our business and harm our financial condition and operating results.
 - If conflicts arise between us and our collaborators or strategic partners, these parties may act in a manner adverse to us and could limit our ability to implement our strategies.
 - RTW Investments, LP, our largest stockholder, may have the ability to significantly influence matters submitted to stockholders for approval.

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[Table Future sales of Contents](#)our common stock in the public market could cause the market price of our common stock to drop significantly, even if our business is performing well.

- If we fail to maintain proper and effective internal control over financial reporting, our ability to produce accurate and timely financial statements could be impaired, which could harm our operating results, investors' views of us and, as a result, the value of our common stock.
- Our internal computer systems, or those of our third-party collaborators or other contractors, may fail or suffer security breaches, which could result in a material disruption of our development programs.
- Unfavorable national or global economic conditions or political developments could adversely affect our business, financial condition or results of operations.

The summary risk factors described above should be read together with the text of the full risk factors below and in the other information set forth in this Annual Report, including our consolidated financial statements and the related notes, as well as in other documents that we file with the [SEC](#). [Securities and Exchange Commission \("SEC"\)](#). If any such risks and uncertainties actually occur, our business, prospects, financial condition, and results of operations could be materially and adversely affected. The risks summarized above or described in full elsewhere in this Annual Report are not the only risks that we face. Additional risks and uncertainties not currently known to us, or that we currently deem to be immaterial may also materially adversely affect our business, prospects, financial condition, and results of operations.

PART I

Item 1. Business

Item 1. Business

Overview

We are a [clinical-stage](#), [multi-platform](#) [fully integrated](#), [late-stage](#) biotechnology company focused on the development of first, only and [best-in-class](#) [best in class](#) gene therapies, with direct on-target mechanism of action and clear clinical endpoints, for rare and devastating diseases. We have three clinical-stage *ex vivo* lentiviral vector ("LV") programs. These programs, which include programs for for:

- Fanconi Anemia ("FA"), a genetic defect in the bone marrow that reduces production of blood cells or promotes the production of faulty blood cells, cells;
- Leukocyte Adhesion Deficiency-I ("LAD-I"), a genetic disorder that causes the immune system to malfunction malfunction; and
- Pyruvate Kinase Deficiency ("PKD"), a [rare](#) red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia. Of these, both

In September 2023, the [FDA](#) accepted the [Biologics License Application \("BLA"\)](#) and granted priority review for RP-L201 for the treatment of severe LAD-I. Treatments in the FA Phase 2 FA program and the Phase ½ LAD-I program are studies were completed in potentially registration-enabling studies 2023 with regulatory filings in the United States ("U.S.") and Europe ("EU"), for FA anticipated in 2024. Additional work on a gene therapy program for the less common FA subtypes C and G is ongoing.

In the U.S., we also have [a two](#) [clinical stage](#) and [one](#) [pre-clinical stage](#) *in vivo* adeno-associated virus ("AAV") program for programs, which include programs for:

- Danon disease ("DD"), a multi-organ lysosomal-associated disorder leading to early death due to heart failure. The [Danon](#) [DD](#) program is currently in an ongoing Phase 1/2 trial. Additionally, we have an AAV vector program targeting
- Plakophilin-2 Arrhythmogenic Cardiomyopathy ("PKP2-ACM"), an inheritable cardiac disorder that is

characterized by a progressive loss of cardiac muscle mass, severe right ventricular dilation, dysplasia, fibrofatty replacement of the myocardium and a high propensity to arrhythmias and sudden death. As This program received FDA clearance of an Investigational New Drug ("IND") application and we have initiated a result of our acquisition of Renovacor, Inc. ("Renovacor"), we are now able to utilize recombinant AAV9-based gene therapy designed to slow or halt progression of Phase 1 study.

- BAG3 Dilated Cardiomyopathy ("DCM"), which is the most common form of cardiomyopathy and is characterized by progressive thinning of the walls of the heart resulting in enlarged heart chambers that are unable to pump blood. Our program utilizes recombinant AAV9-based gene therapy designed to slow or halt progression of BAG3-DCM.

We have global commercialization and development rights to all of these product candidates under royalty-bearing license agreements.

Effective December 2021, a decision was made to no longer pursue Rocket-sponsored clinical evaluation of RP-L401; this program was returned to academic innovators. Although we believe that gene therapy may be beneficial to patients afflicted with this disorder, we have opted to focus available resources towards advancement of RP-A601, RP-A501, RP-L102, RP-L201, RP-L301, and BAG3-DCM based on the compelling clinical data to date and potential for therapeutic advancement in these severe disorders of childhood and young adulthood.

Gene Therapy Overview

Genes are composed of sequences of deoxyribonucleic acid ("DNA"), which provide the code for proteins that perform a broad range of physiologic functions in all living organisms. Although genes are passed on from generation to generation, genetic changes, also known as mutations, can occur in this process. These changes can result in the lack of production of proteins or the production of altered proteins with reduced or abnormal function, which can in turn result in disease.

Gene therapy is a therapeutic approach in which an isolated gene sequence or segment of DNA is administered to a patient, most commonly for the purpose of treating a genetic disease that is caused by genetic mutations. Currently available therapies for many genetic diseases focus on administration of large proteins or enzymes and typically address only the symptoms of the disease. Gene therapy aims to address the disease-causing effects of absent or dysfunctional genes by delivering functional copies of the gene sequence directly into the patient's cells, offering the potential for curing the genetic disease, rather than simply addressing symptoms.

We are using modified non-pathogenic viruses for the development of our gene therapy treatments. Viruses are particularly well suited as delivery vehicles because they are adept at penetrating cells and delivering genetic material inside a cell. In creating our viral delivery vehicles, the viral (pathogenic) genes are removed and are replaced with a functional form of the missing or mutant gene that is the cause of the patient's genetic disease. The functional form of a missing or mutant gene is called a therapeutic gene, or the "transgene." The process of inserting the transgene is called "transduction." Once a virus is modified by replacement of the viral genes with a transgene, the modified virus is called a "viral vector." The viral vector delivers the transgene into the targeted tissue or organ (such as the cells inside a patient's bone marrow). We have two types of viral vectors in development, LV and AAV. We believe that our LV and AAV-based programs have the potential to offer a significant and long-lasting therapeutic benefit to patients that is durable (long-lasting) patients.

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The gene therapies can be delivered either (1) *ex vivo* (outside the body), in which case the patient's cells are extracted and the vector is delivered to these cells in a controlled, safe laboratory setting, with the modified cells then being reinserted into the patient, or (2) *in vivo* (inside the body), in which case the vector is injected directly into the patient, either intravenously ("IV") or directly into a specific tissue at a targeted site, with the aim of the vector delivering the transgene to the targeted cells.

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We believe that scientific advances, clinical progress, and the greater regulatory acceptance of gene therapy have created a promising environment to advance gene therapy products as these products are being designed to restore cell function and improve clinical outcomes, which in many cases include prevention of death at an early age. The FDA approval of several gene therapies in recent years indicates that there is a regulatory pathway forward for gene therapy products.

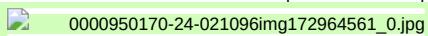
Essential Terminology

Set forth below is an abbreviated index of certain key terms and optimal ranges of values used in the discussion of LV and AAV gene therapies.

Term	Definition	Optimal Ranges
LV Therapy (hematopoietic disorders)		
CD34+ cell(s)	Hematopoietic Stem Cell (most CD34+ cells are not true stem cells, but this continues to be the most clinically useful measure)	Will depend on underlying disorder, generally > 1 million CD34+ cells/kg.
Vector copy number (VCN) [product]	The average number of gene copies per infused stem cell (as determined by DNA analysis; this is an average ratio, not a precise value)	0.5 to 2 has been target in some LV clinical studies (5.0 generally considered maximum)
Vector copy number (VCN) [in vivo, post-treatment]	The average number of gene copies per peripheral blood or bone marrow cell (as determined by DNA analysis; this is an average ratio, not a precise value)	Will depend on underlying disorder, but many disorders may be correctable with in vivo VCNs << 1.0
AAV Therapy		
Vector copy number (VCN) [in vivo, post-treatment]	The average number of gene copies per cell in the organ of interest (as determined by DNA analysis; this is an average ratio, not a precise value)	Will depend on underlying disorder, but vivo VCNs << 1.0

Pipeline Overview

The chart below shows the current phases of development of **Rocket's**our programs and product candidates:



Cardiovascular Programs

Danon Disease

Danon disease ("DD")

DD is a multi-organ lysosomal-associated disorder leading to early death due to heart failure. DD is caused by mutations in the gene encoding lysosome-associated membrane protein 2 ("LAMP-2"), a mediator of autophagy. This mutation results in the accumulation of autophagic vacuoles, predominantly in cardiac and skeletal muscle. Male patients often require heart transplantation and typically die in their teens or twenties from progressive heart failure. Along with severe cardiomyopathy, other DD-related manifestations can include skeletal muscle weakness and intellectual impairment. There are no specific therapies available for the treatment of DD and medications typically utilized for the treatment of congestive heart failure ("CHF") are not believed to modify progression to end-stage CHF. Patients with end-stage CHF may undergo heart transplant, which currently is available to a minority of patients, is associated with significant short- and long-term complications and is not curative of the disorder in the long-term. RP-A501 is in clinical trials as an *in vivo*therapy for DD, which is estimated to have a prevalence of 15,000 to 30,000 patients in the U.S. and the EU.

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DD is an X-linked dominant, monogenic rare inherited disorder characterized by progressive cardiomyopathy which is almost universally fatal in males even in settings where cardiac transplantation is available. DD predominantly affects males early in life and is characterized by absence of *LAMP2B* expression in the heart and other tissues. Preclinical models of DD have demonstrated that AAV-mediated transduction of the heart results in reconstitution of *LAMP2B* expression and improvement in cardiac function.

We currently have one adeno-associated viral vector AAV program targeting DD, RP-A501. We have treated seven patients in the RP-A501 Phase 1 clinical trial, which enrolled adult/older adolescent and pediatric male DD patients. This includes a first cohort evaluating a low-dose (6.7e13 genome copies (gc)/kilogram (kg)) in adult/older adolescent patients aged 15 or greater (n=3), a second cohort evaluating a higher dose (1.1e14 gc/kg) in adult/older adolescent patients aged 15 or greater (n=2), and a pediatric cohort at a low dose level (6.7e13 gc/kg; kg; n=2).

As previously disclosed, a patient receiving therapy on the high dose cohort (1.1e14 gc/kg dose) had progressive heart failure and underwent a heart transplant at month five following therapy. This patient had more advanced disease than the four other adult/older adolescent patients who received treatment in the low and high dose cohorts, as evidenced by diminished baseline left ventricle ("LV") ejection fraction (35%) on echocardiogram and markedly elevated LV left ventricle filling pressure prior to treatment. The patient's clinical course was characteristic of DD progression. The patient is doing well post-transplant.

Based on the initial efficacy observed in the low dose cohort and to mitigate complement-mediated safety concerns observed in the high dose cohort (thrombotic microangiopathy ("TMA")) and in agreement with the FDA, we are focusing on the low dose (6.7e13 gc/kg) and we will no longer administer doses of 1.1e14 gc/kg or higher in this trial. Additional safety measures have been implemented and are reflected in the updated trial protocol. These measures include exclusion of patients with end-stage heart failure, and a refined immunomodulatory regimen involving transient B- and T-cell mediated inhibition, with emphasis on preventing complement activation, while also enabling lower steroid doses and earlier steroid taper, with all immunosuppressive therapy discontinued 2-3 months following administration of RP-A501.

We are conducting conducted a variety of efficacy assessments in the Phase I clinical study to measure the prospect of benefit for patients. These assessments include included the following:

- New York Heart Association ("NYHA") Functional Classification is the most commonly used heart failure classification system. NYHA Class II is where a patient exhibits a slight limitation of physical activity, is comfortable at rest, and ordinary physical activity results in fatigue, palpitation and/or dyspnea. Class I is where a patient exhibits no limitation of physical activity and ordinary physical activity does not cause undue fatigue, palpitation and/or dyspnea. Class III and IV are considered more severe or advanced heart failure.
- Brain natriuretic peptide ("BNP") is a blood-based evaluation and a key marker of heart failure with prognostic significance in CHF and cardiomyopathies. Elevations in BNP are strongly associated with worsening heart failure and poor outcomes in cardiovascular disease.

- High sensitivity troponin I ("hsTnI") is a blood-based evaluation and a key marker of cardiac injury, one that is (like BNP) frequently elevated in DD patients and has been shown to be markedly elevated in patients with advanced stage disease.
- Echocardiographic measurements of heart thickness, most notably, left ventricular mass ("LVM") and maximal left ventricular wall thickness, ("MLVWT"), indicate the degree of hypertrophy present in the heart.
- Kansas City Cardiovascular Questionnaire ("KCCQ") is a validated, patient-reported outcomes assessment that measures a patients perception of their heart failure symptoms, impact of disease on physical and social function, and the impact of their heart failure on overall health status and quality of life. Assessment scores range from 0 (very poor health status) to 100 (excellent health status). Changes in KCCQ score of +/- 5 points are considered meaningful and have been shown to correlate with outcomes.

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- Histologic examination of endomyocardial biopsies via hematoxylin and eosin ("H&E") histology and electron microscopy is used to detect evidence of DD-associated tissue derangements, including the presence of autophagic vacuoles and disruption of myofibrillar architecture, each of which are characteristic of DD-related myocardial damage.
- LAMP2B gene expression in endomyocardial biopsy samples is measured via both immunohistochemistry and Western blot and confirms the presence of LAMP2B protein in DD cardiac tissue following RP-A501 treatment.

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