
**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): March 6, 2018

Rocket Pharmaceuticals, Inc.
(Exact Name of Registrant as Specified in its Charter)

Delaware
(State or Other Jurisdiction
of Incorporation)

001-36829
(Commission
File Number)

04-3475813
(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 2.02 Results of Operation and Financial Condition.

On March 6, 2018, Rocket Pharmaceuticals, Inc. (the “Company”) announced its financial results for the year ended December 31, 2017. A copy of the press release is being furnished as Exhibit 99.1 to this Current Report on Form 8-K.

The information in this Report on Form 8-K and Exhibit 99.1 attached hereto is intended to be furnished and shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934 (the “Exchange Act”) or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933 or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 9.01 Financial Statements and Exhibits

(a) *Financial statements of businesses acquired.*

The audited financial statements of Rocket Pharmaceuticals, Ltd., which comprise the balance sheets as of December 31, 2017 and 2016, and the related Statements of Operations, Shareholders’ Equity and Cash Flows for the years ended December 31, 2017 and December 31, 2016, and the related notes thereto, are filed as Exhibit 99.2 to this Current Report on Form 8-K.

(d) *Exhibits*

Exhibit No.	Description
23.1	Consent of EisnerAmper LLP
99.1	Press Release of the Company, dated March 6, 2018
99.2	Audited Financial Statements of Rocket Pharmaceuticals, Ltd. as of and for the years ended December 31, 2017 and 2016

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.

Date: March 7, 2018

Rocket Pharmaceuticals, Inc.

By: /s/ Gaurav Shah

Name: Gaurav Shah

Title: President and Chief Executive Officer

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We consent to the incorporation by reference in the Registration Statements of Rocket Pharmaceuticals, Inc. (formerly Inotek Pharmaceuticals Corporation) on Form S-3 (No. 333-210586) and Forms S-8 (Nos. 333-204501, 333-212308 and 333-216892) of our report dated March 7, 2018, on our audits of the financial statements of Rocket Pharmaceutical, Ltd. as of December 31, 2017 and 2016 and for each of the years then ended which report is included in this Form 8-K to be filed on or about March 7, 2018.

/s/ EISNERAMPER LLP

EISNERAMPER LLP
New York, New York
March 7, 2018



Rocket Pharmaceuticals Reports Full Year 2017 Financial Results and Operational Highlights

– Enters 2018 as a Public Company with Solid Financial Position Following Successful Completion of Merger with Inotek Pharmaceuticals and Subsequent Follow-on Offering –

– Multiple Milestones Expected in 2018, with Additional Data in Fanconi Anemia, Disclosure of AAV Program, and Several Programs Advancing Toward the Clinic –

NEW YORK—March 6, 2018—Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) (“Rocket”), a leading U.S.-based multi-platform gene therapy company, today reported financial results and operational highlights for the full year ended December 31, 2017.

“2017 was an important year for Rocket. We advanced our pipeline of gene therapy programs focused on life-threatening and devastating pediatric diseases, and we implemented strategic initiatives that have strengthened our organization,” said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. “We believe Rocket’s multi-platform development approach uniquely positions us to aggressively pursue a broad range of best-in-class indications, enabling first-mover advantage.”

“With the successful closing of our merger with Inotek Pharmaceuticals and recent public offering in January, we enter 2018 with a solid financial position to accomplish our key clinical and operational goals,” continued Dr. Shah. “In addition to more clinical data on our lead program in Fanconi Anemia (FA), we plan to announce the disease indication for our adeno-associated viral vector (AAV) gene therapy program in the second half of this year. We also expect to file Investigational Medicinal Product Dossier (IMPD) applications for our Leukocyte Adhesion Deficiency-I (LAD-I) and Pyruvate Kinase Deficiency (PKD) programs, as well as an Investigational New Drug (IND) application for our AAV program over the next 12 months. With multiple programs moving forward in parallel, we believe we have the key elements in place to build a transformative, multi-platform gene therapy company delivering sustained, long-term growth for our shareholders.”

Recent Pipeline and Corporate Updates

- **Promising preliminary patient data reported for RP-L102, a Phase 1/2 lentiviral vector (LVV)-based gene therapy for FA.** At the European Society of Gene and Cell Therapy’s Annual Meeting in October 2017, Rocket’s collaboration partners at the Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT) in Spain presented interim results from the ongoing Phase 1/2 trial of RP-L102 in FA. Data presented in FA patients, without the use of conditioning, showed promising *in vivo* engraftment and stabilization of blood counts despite declining counts in the months to years preceding therapy.
- **Studies of FA gene therapy product, RP-L101, utilizing new gene modified cell product manufacturing process underway.** At the American Society of Hematology Annual Meeting in December 2017, Rocket’s collaboration partners at the Fred Hutchinson Cancer Research Center presented data from a pilot clinical trial assessing the feasibility of a novel lineage depletion protocol for hematopoietic stem cell (HSC) enrichment and subsequent transduction with the LVV-based gene therapy, RP-L101. Collection, lentiviral transduction and re-infusion of gene



modified cells was shown to effectively produce long-term engraftment in mice. Patient treatment with this novel protocol is underway.

- **Three additional LVV-based gene therapy pipeline programs in rare bone marrow disorders advance towards the clinic.** LAD-I and PKD programs continue to move forward in preclinical IND-enabling studies. An additional program targeting Infantile Malignant Osteopetrosis (IMO) is also in preclinical development. Similar to FA, these diseases can lead to serious complications and have very limited long-term safe and effective treatment options.
- **Rocket commenced trading on the NASDAQ Global Market under the symbol “RCKT”, following the successful closing of its with merger with Inotek Pharmaceuticals Corporation (Inotek) in January.** In conjunction with the completion of the merger, the Company announced its executive management team will be led by Dr. Shah and will consist of: Jonathan Schwartz, M.D., Chief Medical Officer, and Kinnari Patel, Pharm.D., MBA, Chief Operating Officer.
- **Rocket completed an oversubscribed underwritten public offering of 6,325,000 shares of its common stock in January 2018.** Net proceeds to Rocket from the offering were approximately \$78.8 million after deducting underwriting discounts and commissions.

Anticipated Milestones

- Updated FA patient data (2Q18)
- Preclinical data and disclosure of the AAV-based gene therapy program (2H18)
- Target rolling IMPD filing for the LAD-I program (4Q18)
- Target IND filing for the AAV-based program (next 12 months)
- Target rolling IMPD filing for the PKD program (next 12 months)

Full Year Rocket Pharmaceuticals Ltd. 2017 Financial Results

- The foregoing financial results presented for the year ended December 31, 2017 represent Rocket Pharmaceuticals Ltd. as a standalone private company, as the merger with Inotek closed on January 4, 2018. The financial statements for the first quarter 2018 will reflect the financial results as a merged public company.
- Research and development expenses were \$14.9 million for the year ended December 31, 2017, compared to \$6.0 million for the year ended December 31, 2016. General and administrative expenses were \$4.9 million for the year ended December 31, 2017, compared to \$1.6 million for the year ended December 31, 2016.
- Net loss was \$19.6 million or \$(219.49) per share (basic and diluted) for the year ended December 31, 2017, compared to \$7.6 million or \$(84.43) per share (basic and diluted) for the year ended December 31, 2016.
- Cash as of December 31, 2017 for Rocket Pharmaceuticals Ltd. was \$18.1 million. Following the merger with Inotek and the January 2018 offering of common stock, cash, cash equivalents and short-term investments as of February 28, 2018 totaled approximately \$185 million, which includes a \$52 million fully convertible debenture which expires in 2021.
- The standalone historical financial statements of Inotek for the year ended December 31, 2017 are presented in Rocket’s Annual Report on Form 10-K for the year ended December 31, 2017.

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) (“Rocket”) is an emerging, clinical-stage biotechnology company focused on developing first-in-class gene therapy treatment options for rare, devastating diseases. Rocket’s multi-platform development approach applies the well-established lentiviral vector



(LVV) and adeno-associated viral vector (AAV) gene therapy platforms. Rocket's lead clinical program is a LVV-based gene therapy for the treatment of Fanconi Anemia (FA), a difficult to treat genetic disease that leads to bone marrow failure and potentially cancer. Preclinical studies of additional bone marrow-derived disorders are ongoing and target Pyruvate Kinase Deficiency (PKD), Leukocyte Adhesion Deficiency-I (LAD-I) and Infantile Malignant Osteopetrosis (IMO). Rocket is also developing an AAV-based gene therapy program for an undisclosed rare pediatric disease. For more information about Rocket, please visit www.rocketpharma.com.

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 products 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, 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.

Contact:

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Opinion on the Financial Statements

We have audited the accompanying balance sheets of Rocket Pharmaceuticals, Ltd. (the “Company”) as of December 31, 2017 and 2016 and the related statements of operations, shareholders’ equity, and cash flows for each of the years then ended and the related notes (collectively referred to as the “financial statements”). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2017 and 2016, and the results of its operations and its cash flows for each of the years then ended, in conformity with accounting principles generally accepted in the United States of America.

Basis for Opinion

These financial statements are the responsibility of the Company’s management. Our responsibility is to express an opinion on the Company’s financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (“PCAOB”) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company’s internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ EisnerAmper LLP

We have served as the Company’s auditor since 2016.

EISNERAMPER LLP
New York, New York
March 7, 2018

Rocket Pharmaceuticals, Ltd.
Balance Sheets
(in thousands, except share and per share amounts)

	December 31, 2017	December 31, 2016
Assets		
Current assets:		
Cash	\$ 18,142	\$ 9,460
Prepaid expenses	813	93
Total current assets	18,955	9,553
Property and equipment, net	985	429
Restricted cash	207	205
Total assets	<u>\$ 20,147</u>	<u>\$ 10,187</u>
Liabilities and shareholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	\$ 2,062	\$ 620
Accrued research and development costs	2,459	1,089
Total current liabilities	4,521	1,709
Deferred rent	107	107
Total liabilities	4,628	1,816
Commitments and contingencies		
Shareholders' equity		
Preferred shares, \$0.01 par value, authorized 1,000,000 shares		
Series A convertible preferred shares; 300,000 shares designated as Series A; 128,738 shares issued and outstanding at December 31, 2017 and 2016; liquidation preference of \$16,092 at December 31, 2017	16,060	16,060
Series B convertible preferred shares; 300,000 shares designated as Series B; 126,909 and none issued and outstanding at December 31, 2017 and 2016, respectively; liquidation preference of \$25,445 at December 31, 2017	25,406	—
Ordinary Shares, \$0.01 par value, 4,000,000 shares authorized; 89,199 and 89,699 shares issued and outstanding at December 31, 2017 and 2016, respectively	1	1
Additional paid-in capital	5,407	4,087
Accumulated deficit	(31,355)	(11,777)
Total shareholders' equity	15,519	8,371
Total liabilities and shareholders' equity	<u>\$ 20,147</u>	<u>\$ 10,187</u>

The accompanying notes are an integral part of these financial statements.

Rocket Pharmaceuticals, Ltd.
Statements of Operations
(in thousands, except share and per share amounts)

	Year Ended December 31, 2017	Year Ended December 31, 2016
Revenue	\$ —	\$ —
Operating expenses:		
Research and development	14,917	5,994
General and administrative	4,855	1,580
Total operating expenses	<u>19,772</u>	<u>7,574</u>
Loss from operations	<u>(19,772)</u>	<u>(7,574)</u>
Research and development incentives	192	—
Interest income	<u>2</u>	<u>1</u>
Net loss	<u>\$ (19,578)</u>	<u>\$ (7,573)</u>
Net loss per share attributable to ordinary shareholders—basic and diluted	<u>\$ (219.49)</u>	<u>\$ (84.43)</u>
Weighted-average ordinary shares outstanding—basic and diluted	<u>89,199</u>	<u>89,699</u>

The accompanying notes are an integral part of these financial statements.

Rocket Pharmaceuticals, Ltd.
Statements of Shareholders' Equity
For the years ended December 31, 2017 and 2016
(in thousands except share amounts)

	Series A Convertible Preferred Shares		Series B Convertible Preferred Shares		Ordinary Shares		Additional paid in capital	Accumulated Deficit	Total Shareholders' Equity
	Shares	Amount	Shares	Amount	Shares	Amount			
Balance at December 31, 2015	127,698	\$15,930	—	\$ —	89,699	\$ 1	\$ 3,813	\$ (4,204)	\$ 15,540
Issuance of Series A convertible preferred shares in connection with license agreements	240	30	—	—	—	—	—	—	30
Issuance of Series A convertible preferred shares in connection with license agreements	800	100	—	—	—	—	—	—	100
Share-based compensation	—	—	—	—	—	—	274	—	274
Net loss	—	—	—	—	—	—	—	(7,573)	(7,573)
Balance at December 31, 2016	128,738	16,060	—	—	89,699	1	4,087	(11,777)	8,371
Repurchase of ordinary shares	—	—	—	—	(500)	—	—	—	—
Issuance of Series B convertible preferred shares, net of issuance costs	—	—	126,909	25,406	—	—	—	—	25,406
Share-based compensation	—	—	—	—	—	—	1,320	—	1,320
Net loss	—	—	—	—	—	—	—	(19,578)	(19,578)
Balance at December 31, 2017	<u>128,738</u>	<u>\$16,060</u>	<u>126,909</u>	<u>\$25,406</u>	<u>89,199</u>	<u>\$ 1</u>	<u>\$ 5,407</u>	<u>\$ (31,355)</u>	<u>\$ 15,519</u>

The accompanying notes are an integral part of these financial statements.

Rocket Pharmaceuticals, Ltd.
Statements of Cash Flows
(in thousands)

	Year Ended December 31, 2017	Year Ended December 31, 2016
Operating Activities:		
Net loss	\$ (19,578)	\$ (7,573)
Adjustments to reconcile net loss to net cash used in operating activities:		
Series A preferred shares issued for services	—	100
Depreciation expense	204	67
Share-based compensation expense	1,320	274
Deferred rent	—	107
Changes in operating assets and liabilities:		
Prepaid expenses	(720)	78
Accounts payable and accrued expenses	1,442	355
Accrued research and development costs	1,370	1,089
Net cash used in operating activities	<u>(15,962)</u>	<u>(5,503)</u>
Investing activities:		
Purchases of property and equipment	(760)	(335)
Net cash used in investing activities	<u>(760)</u>	<u>(335)</u>
Financing activities:		
Payments from related party payable	—	(14)
Proceeds from issuance of Series A convertible preferred shares, net	—	30
Proceeds from issuance of Series B convertible preferred shares, net	25,406	—
Net cash provided by financing activities	25,406	16
Net change in cash and restricted cash	8,684	(5,822)
Cash and restricted cash at beginning of year	9,665	15,487
Cash and restricted cash at end of year	<u>\$ 18,349</u>	<u>\$ 9,665</u>

The accompanying notes are an integral part of these financial statements.

Rocket Pharmaceuticals, Ltd.
Notes to Financial Statements
(in thousands, except share and per share amounts)

1. Organization and Nature of Business

Rocket Pharmaceuticals, Ltd. (“Rocket Pharma” or the “Company”) is a multi-platform biotechnology company focused on the development of first-in-class gene therapies for rare and devastating pediatric diseases. Rocket Pharma has two lentiviral virus (“LVV”) programs currently undergoing clinical testing targeting Fanconi Anemia (“FA”), a genetic defect in the bone marrow that reduces production of blood cells or promotes the production of faulty blood cells, and three additional LVV programs targeting other rare genetic diseases. In addition, Rocket Pharma has an adeno-associated virus (“AAV”) program for which it expects to file an investigational new drug (“IND”) application in the next 12 months, which will permit the commencement of human clinical studies thereafter. Rocket Pharma has full global commercialization and development rights to all of its product candidates under royalty-bearing license agreements, with the exception of the CRISPR/Cas9 development program (described below) for which Rocket Pharma currently only has development rights.

Rocket Pharma’s two leading LVV and AAV technology platforms are each being designed in collaboration with leading academic and industry partners. Through its gene therapy platforms, Rocket aims to restore normal cellular function by modifying the defective genes that cause each of the targeted disorders.

On January 4, 2018, Inotek Pharmaceuticals Corporation (“Inotek”) and Rocket Pharma completed a business combination in accordance with the terms of the Agreement and Plan of Merger and Reorganization (the “Merger Agreement”), dated as of September 12, 2017, by and among Inotek, Rome Merger Sub, a wholly owned subsidiary of Inotek (“Merger Sub”) and Rocket Pharma, pursuant to which Merger Sub merged with and into Rocket Pharma, with Rocket Pharma surviving as a wholly owned subsidiary of Inotek. This transaction is referred to as “the Merger.” Immediately following the Merger, Inotek changed its name to Rocket Pharmaceuticals, Inc. (“Rocket”).

As a result of the Merger, each outstanding share of Rocket Pharma share capital (including shares of Rocket Pharma share capital to be issued upon exercise of outstanding share options) automatically converted into the right to receive approximately 76.185 shares of Inotek’s common stock, par value \$0.001 per share. Following the closing of the Merger, holders of Inotek’s common stock immediately prior to the Merger owned approximately 18.643% on a fully diluted basis, and holders of Rocket Pharma common stock immediately prior to the Merger owned approximately 81.357% on a fully diluted basis, of Rocket’s common stock.

In connection with the closing of the Merger, Rocket’s common stock began trading on The NASDAQ Global Market under the ticker symbol “RCKT” on January 5, 2018. The accompanying financial statements do not give effect to the Merger.

2. Risks and Liquidity

The Company has not generated any revenue and has incurred losses since inception. Operations of the Company are subject to certain risks and uncertainties, including, among others, uncertainty of drug candidate development, technological uncertainty, uncertainty regarding patents and proprietary rights, having no commercial manufacturing experience, marketing or sales capability or experience, dependency on key personnel, compliance with government regulations and the need to obtain additional financing. Drug candidates currently under development will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure and extensive compliance-reporting capabilities.

The Company’s drug candidates are in the development stage. There can be no assurance that the Company’s research and development will be successfully completed, that adequate protection for the Company’s intellectual property will be obtained, that any products developed will obtain necessary government approval or that any approved products will be commercially viable. Even if the Company’s product development efforts are successful, it is uncertain when, if ever, the Company will generate significant revenue from product sales. The Company operates in an environment of rapid change in technology and substantial competition from pharmaceutical and biotechnology companies.

Rocket Pharmaceuticals, Ltd.
Notes to Financial Statements
(in thousands, except share and per share amounts)

The Company's financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities in the ordinary course of business. The Company has experienced negative cash flows from operations and had an accumulated deficit of \$31,355 as of December 31, 2017. As of December 31, 2017, the Company had \$18,142 of cash on hand. On January 26, 2018, Rocket closed a public offering of common stock and received net proceeds of approximately \$78.8 million (see Note 15). Rocket expects that its cash on hand as of December 31, 2017 plus the net proceeds of \$78.8 million received from the public offering would be sufficient to fund its operating expenses and capital expenditure requirements through at least March 2019.

In the longer term, the future viability of the Company is dependent on its ability to generate cash from operating activities or to raise additional capital to finance its operations. The Company's failure to raise capital as and when needed could have a negative impact on its financial condition and ability to pursue its business strategies.

3. Summary of Significant Accounting Policies

Use of Estimates

The preparation of the financial statements in conformity with accounting principles generally accepted in the United States ("US GAAP") requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. Significant estimates and assumptions reflected in these financial statements include, but are not limited to, the accrual of research and development expenses and the valuation of equity transactions and share-based awards. Changes in estimates and assumptions are reflected in reported results in the period in which they become known. Actual results could differ from those estimates.

Research and Development

Research and development costs, which include salaries and staff costs, license costs, regulatory and scientific consulting fees, as well as contract research, and share-based compensation expense, are accounted for in accordance with ASC Topic 730, *Research and Development*, ("ASC 730").

The Company does not currently have any commercial biopharmaceutical products, and does not expect to have any for several years, if at all. Accordingly, research and development costs are expensed as incurred. While certain of the Company's research and development costs may have future benefits, the policy of expensing all research and development expenditures is predicated on the fact that the Company has no history of successful commercialization of product candidates to base any estimate of the number of future periods that would be benefited.

Share-Based Compensation

The Company measures the compensation expense of share options and other share-based awards granted to employees and directors using the grant date fair value of the award and recognizes compensation expense as determined by the Black-Scholes Option pricing model on a straight-line basis over their requisite service period, which is generally the vesting period of the respective award.

The Company initially measures the compensation expense of share-based awards granted to consultants using the grant date fair value of the award. Compensation expense is recognized over the period during which services are rendered by such consultants. At the end of each financial reporting period prior to completion of services being rendered, the compensation expense is remeasured using the then current fair value of the share-based award, based on updated assumption inputs in the Black-Scholes option pricing model.

The Company classifies share-based compensation expense in its statement of operations in the same manner in which the award recipient's payroll costs are classified or in which the award recipients' service payments are classified.

The Company recognizes compensation expense for only the portion of awards that are expected to vest. In developing a forfeiture rate estimate, the Company estimated pre-vesting forfeitures for service-based awards. The impact of a forfeiture rate adjustment will be recognized in full in the period of adjustment, and if the actual forfeiture rate is materially different from the Company's estimate, the Company may be required to record adjustments to share-based compensation expense in future periods.

Rocket Pharmaceuticals, Ltd.
Notes to Financial Statements
(in thousands, except share and per share amounts)

The Company lacks company-specific historical and implied volatility information. Therefore, it estimates its expected share volatility based on the historical volatility of a publicly traded set of peer companies. The expected term of the Company's share options has been determined utilizing the "simplified" method for awards that qualify as "plain vanilla" options. The expected term of share options granted to non-employees is equal to the contractual term of the option award. The risk free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future.

Cash and Restricted Cash

Cash is maintained at U.S. financial institutions and, at times, balances may exceed federally insured limits. The Company has never experienced losses related to these balances. Restricted cash consists of a letter of credit under the Company's operating lease. See Note 13. Cash and restricted cash consists of the following at December 31, 2017 and 2016:

	<u>2017</u>	<u>2016</u>
Cash	\$18,142	\$9,460
Restricted cash	207	205
	<u>\$18,349</u>	<u>\$9,665</u>

Property and Equipment

Property and equipment are stated at cost less accumulated depreciation. Depreciation expense is recognized using the straight-line method over the useful life of the asset. The estimated useful lives are three to five years. Expenditures for repairs and maintenance of assets are charged to expense as incurred. Upon retirement or sale, the cost and related accumulated depreciation of assets disposed of are removed from the accounts and any resulting gain or loss is included in loss from operations. If the carrying amount of the assets or asset group is not recoverable on an undiscounted cash flow basis, impairment is recognized to the extent that the carrying value exceeds its fair value. No impairment losses were recognized during the years ended December 31, 2017 and 2016.

Fair Value of Financial Instruments

The carrying amounts of cash, accounts payable, accrued expenses and accrued research and development costs approximate their fair values due to their short-term nature.

Income Taxes

The Company accounts for income taxes under the asset and liability method. The Company recognizes deferred tax assets and liabilities for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases, as well as for operating loss and tax credit carry-forwards. The Company measures deferred tax assets and liabilities using enacted tax rates expected to apply to taxable income in the years in which the Company expects to recover or settle those temporary differences. The Company recognizes the effect of a change in tax rates on deferred tax assets and liabilities in the results of operations in the period that includes the enactment date. The Company reduces the measurement of a deferred tax asset, if necessary, by a valuation allowance if it is more likely than not that the Company will not realize some or all of the deferred tax asset.

The Company accounts for uncertainty in income taxes recognized in the financial statements by applying a two-step process to determine the amount of tax benefit to be recognized. First, the tax position must be evaluated to determine the likelihood that it will be sustained upon external examination by the taxing authorities. If the tax position is deemed more-likely-than-not to be sustained, the tax position is then assessed to determine the amount of benefit to recognize in the financial statements. The amount of the benefit that may be recognized is the largest amount that has a greater than 50% likelihood of being realized upon ultimate settlement. The provision for income taxes includes the effects of any resulting tax reserves, or unrecognized tax benefits, that are considered appropriate as well as the related net interest and penalties. For discussion and impact of the Tax Cut and Jobs Act (the "Act"), see Note 9.

Deferred Rent Expense

The Company recognizes rent expense on a straight-line basis, after considering the effect of rent escalation provisions resulting in a level rent expense recognized over the lease term.

Rocket Pharmaceuticals, Ltd.
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Foreign currency transactions

Certain transactions in 2017 and 2016 are denominated in Euros. Gains and losses on foreign currency transactions is not significant for the years ended December 31, 2017 and 2016.

Comprehensive Loss

Comprehensive loss is equal to net loss for all periods presented.

NYC Biotechnology Tax Credit Program

New York City allows investors and owners of emerging technology companies focused on biotechnology to claim a tax credit against the General Corporation Tax and Unincorporated Business Tax for amounts paid or incurred for certain facilities, operations, and employee training in New York City. During the year ended December 31, 2017, the Company received a payment of \$192 from New York City in connection with this program. The payment is recorded as research and development incentives in the Company's statements of operations. The credit is recognized as income when notified by New York City of the eligibility for the credit and the credit amount.

Net Loss per Share

The Company follows the two-class method when computing net income (loss) per share as the Company has issued shares that meet the definition of participating securities. The two-class method determines net income (loss) per share for each class of ordinary shares and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income available to ordinary shareholders for the period to be allocated between ordinary shares and participating securities based upon their respective rights to receive dividends as if all income for the period had been distributed.

Basic net income (loss) per share attributable to ordinary shareholders is computed by dividing the net income (loss) attributable to ordinary shareholders by the weighted average number of ordinary shares outstanding for the period. Diluted net income (loss) attributable to ordinary shareholders is computed by adjusting net income (loss) attributable to ordinary shareholders to reallocate undistributed earnings based on the potential impact of dilutive securities. Diluted net income (loss) per share attributable to ordinary shareholders is computed by dividing the diluted net income (loss) attributable to ordinary shareholders by the weighted average number of ordinary shares outstanding for the period, including potential dilutive ordinary shares. For purpose of this calculation, outstanding options, warrants to purchase ordinary shares, convertible preferred shares and contingently issuable equity are considered potential dilutive ordinary shares.

The Company's convertible preferred shares contractually entitled the holders of such shares to participate in dividends but contractually did not require the holders of such shares to participate in losses of the Company. Accordingly, in periods in which the Company reports a net loss attributable to ordinary shareholders, diluted net loss per share attributable to ordinary shareholders is the same as basic net loss per share attributable to ordinary shareholders, since dilutive ordinary shares are not assumed to have been issued if their effect is anti-dilutive.

Recent Accounting Pronouncements

In January 2017, the Financial Accounting Standards Board ("FASB") issued ASU No. 2017-01, *Business Combinations (Topic 805): Clarifying the Definition of a Business* ("ASU 2017-01"). The amendments in this update clarify the definition of a business with the objective of adding guidance to assist entities with evaluating whether transactions should be accounted for as acquisitions or disposals of assets or businesses. The definition of a business affects many areas of accounting including acquisitions, disposals, goodwill and consolidation. The standard is effective for annual periods beginning after December 15, 2017. This pronouncement may change the Company's accounting for future acquisitions.

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In November 2016, the FASB issued ASU 2016-18, *Restricted Cash*, which clarifies guidance and presentation related to restricted cash in the statement of cash flows, including stating that restricted cash should be included within cash and cash equivalents on the statement of cash flows. The standard is to be applied retrospectively for all companies. The Company adopted this guidance as of December 31, 2017. The Company has reclassified the restricted cash in the statement of cash flows for the year ended December 31, 2016 and disclosed the reconciliation of the amounts of cash and restricted cash reported on the balance sheets to the totals in the statement of cash flows as of December 31, 2017 and 2016.

In February 2016, the FASB issued ASU 2016-02, *Leases (Topic 842)*. The new standard establishes a right-of-use (“ROU”) model that requires a lessee to record a ROU asset and a lease liability on the balance sheet for all leases with terms longer than 12 months. Leases will be classified as either finance or operating, with classification affecting the pattern of expense recognition in the income statement. ASU 2016-02 is effective for public companies for annual periods beginning after December 15, 2018, including interim periods within those annual periods, with early adoption permitted. A modified retrospective transition approach is required for lessees for capital and operating leases existing at, or entered into after, the beginning of the earliest comparative period presented in the financial statements, with certain practical expedients available. The Company is currently evaluating the potential impact of the adoption of this standard.

4. Property and Equipment, Net

Property and equipment, net consisted of the following:

	December 31, 2017	December 31, 2016
Laboratory equipment	\$ 1,042	\$ 306
Computer equipment	98	78
Furniture and fixtures	115	111
	1,255	495
Less: Accumulated depreciation	(270)	(66)
	<u>\$ 985</u>	<u>\$ 429</u>

Depreciation expense was \$204 and \$66 for the years ended December 31, 2017 and 2016, respectively.

5. Convertible Preferred Shares

As of December 31, 2017 and 2016, the Company’s certificate of incorporation, as amended and restated, authorized the Company to issue up to 1,000,000 shares of \$0.01 par value Preferred Shares.

In February 2017, the Company entered into a Series B Preferred Shares (“Series B”) purchase agreement with multiple purchasers pursuant to which the Company was authorized to sell and issue up to 300,000 shares of Series B for \$200.50 per share. The Company sold an aggregate of 126,909 shares of Series B for net proceeds of approximately \$25,406.

During the year ended December 31, 2016, the Company issued 800 shares of Series A Preferred Shares (“Series A”) to Hutch under a licensing agreement for \$100. The Company also issued 240 shares of Series A to private investors at \$125 per share for a total of \$30. In connection with the Merger with Inotek which closed on January 4, 2018, all of the Series A and Series B of Rocket Pharma were converted into Rocket common stock. This conversion was in accordance with the original terms. See Note 1.

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6. Ordinary Shares

Each ordinary share entitles the holder to one vote on all matters submitted to a vote of the Company's shareholders. Ordinary shareholders are entitled to receive dividends, as may be declared by the board of directors, if any, subject to the preferred dividend rights of the Series A. When dividends are declared on ordinary shares, the Company must declare at the same time a dividend payable to the holders of Series A equivalent to the dividend amount they would receive if each preferred share were converted into an ordinary share. The Company may not pay dividends to ordinary shareholder until all dividends declared but unpaid on the Preferred Shares have been paid in full. The Company has never declared a cash dividend. During the year ended December 31, 2017, the Company repurchased 500 ordinary shares from an investor at a price of \$0.01 per share.

As of December 31, 2017 and 2016, the Company had reserved 385,647 and 258,738 shares, respectively, for the conversion of the outstanding shares of Series A and Series B Preferred Shares (see Note 5), the exercise of outstanding share options and the number of shares remaining available for future grant under the Company's 2015 Share Option Plan (see Note 7).

7. Share-Based Awards

2015 Share Option Plan

The Company's 2015 Share Option Plan provides for the Company to grant incentive stock options or nonqualified stock options for the purchase of ordinary shares to employees, members of the board of directors and consultants. The 2015 Share Option Plan is administered by an administrative committee appointed by the board of directors or, in the absence of such appointment, the entire board of directors. The exercise prices, vesting and other restrictions are determined at the discretion of the board of directors, or their committee if so delegated, except that the exercise price per share of share options may not be less than 100% of the fair market value of the share of ordinary shares on the date of grant (or 110% of the fair market value in the case of an employee who owns shares representing more than 10% of the voting power of all classes of shares for the Company) and the term of share options may not be greater than ten years (or five years in the case of an employee who owns shares representing more than 10% of the voting power of all classes of shares for the Company). The Company generally grants share-based awards with service conditions only ("service-based" awards).

The total number of ordinary shares that may be issued under the 2015 Share Option Plan was 130,000 shares.

As required by the 2015 Share Option Plan, the exercise price for share options granted was not to be less than the fair value of ordinary shares as determined by the Company as of the date of grant. The Company valued its ordinary shares by taking into consideration its most recently available valuation of ordinary shares performed by management and the board of directors as well as additional factors which may have changed since the date of the most recent contemporaneous valuation through the date of grant.

Share Option Valuation

The weighted average assumptions that the Company used in the Black-Scholes pricing model to determine the fair value of the share options granted to employees and directors were as follows:

	<u>Year Ended</u> <u>December 31, 2017</u>	<u>Year Ended</u> <u>December 31, 2016</u>
Risk-free interest rate	2.06%	1.65%
Expected dividend yield	0.0%	0.0%
Expected stock price volatility	91.29%	96.10%
Expected term of options (in years)	6.00	6.00
Expected forfeiture rate	0.0%	0.0%

The Company recognizes compensation expense for only the portion of awards that are expected to vest.

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Share Options

The following table summarizes share option activity under the 2015 Share Option Plan from December 31, 2015 to December 31, 2017:

	Number of Shares	Weighted- Average Exercise Price	Weighted- Average Remaining Contractual Term (in years)
Outstanding at December 31, 2015	70,000	\$ 52.51	9.9
Granted	13,203	91.89	
Exercised	—	—	
Forfeited	(1,925)	91.89	9.13
Outstanding at December 31, 2016	81,278	60.02	9.0
Granted	10,670	226.75	
Exercised	—	—	
Forfeited	(600)	91.89	8.88
Outstanding at December 31, 2017	<u>91,348</u>	\$ 77.75	8.17
Options exercisable at December 31, 2017	<u>73,443</u>	\$ 55.55	7.95

The weighted average grant date fair value of share options granted during the years ended December 31, 2017 and 2016 was \$169.74 and \$74.15, respectively.

Share-Based Compensation

The Company recorded \$1,320 and \$274 of share-based compensation expense as research and development and general and administrative expense in its statements of operations for the years ended December 31, 2017 and 2016, respectively. Share-based compensation research and development expense was \$523 and \$159 and general and administrative expense was \$797 and \$115 for the years ended December 31, 2017 and 2016, respectively.

As of December 31, 2017, the Company had an aggregate of \$2,607 of unrecognized share-based compensation cost, which is expected to be recognized over the weighted average period of 2.46 years.

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8. Net Loss Per Share

Basic and diluted net loss per share attributable to ordinary shareholders was calculated as follows:

	Year Ended December 31, 2017	Year Ended December 31, 2016
Numerator:		
Net loss attributable to ordinary shareholders	\$ (19,578)	\$ (7,573)
Denominator:		
Weighted-average ordinary shares outstanding—basic and diluted	89,199	89,699
Net loss per share attributable to ordinary shareholders—basic and diluted	\$ (219.49)	\$ (84.42)

The Company excluded the following potential ordinary shares, presented based on amounts outstanding at each period end, from the computation of diluted net loss per share attributable to ordinary shareholders for the periods indicated because including them would have had an anti-dilutive effect:

	December 31, 2017	December 31, 2016
Options to purchase ordinary shares	91,348	81,278
Redeemable Series A convertible preferred shares (as converted to ordinary shares)	128,738	128,738
Redeemable Series B convertible preferred shares (as converted to ordinary shares)	126,909	—
	<u>346,995</u>	<u>210,016</u>

9. Income Taxes

The Company is not subject to tax in its country of incorporation and is only subject to tax in the United States on its income that is effectively connected to a U.S. trade or business. As of and for the years ended December 31, 2017 and 2016, the Company has determined that its activities in the U.S. do not rise to the level of a U.S. trade or business for U.S. federal income tax purposes; and therefore, it has not recorded a current or deferred federal income tax expense or benefit since its inception. The Company's loss before income taxes was \$(19,578) and \$(7,573) for the years ended December 31, 2017 and 2016, respectively, and was generated entirely in New York City. The Company is subject to tax for state and local purposes in the U.S.; however, due to the fact that the Company has reported losses since inception it has not recorded a current or net deferred income tax expense or benefit.

A reconciliation of income tax benefit computed at the statutory federal income tax rate to income taxes as reflected in the financial statements is as follows:

	For the year ended December 31, 2017	For the year ended December 31, 2016
U.S. federal tax at statutory rate	34%	34%
Foreign source income not subject to tax	(34%)	(34%)
New York City tax	6.5%	6.3%
Valuation allowance	(6.5%)	(6.3%)
Effective tax rate	<u>0%</u>	<u>0%</u>

Deferred taxes are recognized for temporary differences between the basis of assets and liabilities for financial statement and income tax purposes. The Company has not recorded any deferred assets or liabilities for US federal income tax purposes. Due to the Company's cumulative history of losses, the Company has determined that it is more likely that not that it will not realize any deferred tax assets for local income tax purposes and has established a valuation allowance against these deferred tax assets.

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The significant components of the Company's deferred income tax assets and liabilities after applying the enacted corporate tax rates are as follows:

	For the year ended December 31, 2017	For the year ended December 31, 2016
Deferred income tax assets (liabilities)		
New York City operating losses carried forward	\$ 1,620	\$ 458
Other	136	25
Valuation allowance	(1,756)	(483)
Net deferred income tax assets	<u>\$ —</u>	<u>\$ —</u>

As of December 31, 2017, the Company has accumulated net operating losses of approximately \$24,789 for New York City tax purposes, which may be available to carry forward and offset future years' taxable income. The losses expire in various amounts starting in 2035.

On December 22, 2017, the Act, was signed into law by the President of the United States. The Act includes a number of provisions, including the lowering of the U.S. corporate tax rate from 34 percent to 21 percent, effective January 1, 2018 and the establishment of a territorial-style system for taxing foreign-source income of domestic multinational corporations. The Company is in the process of quantifying the tax impacts of The Act, but at this time does not believe the provisions will have a material impact on the Company's financial reporting. The Company will continue to monitor and quantify the impact of the Act and will record any adjustments in accordance with the guidance in Staff Accounting Bulletin No. 118.

As of December 31, 2017, the Company had no unrecognized tax benefits or liabilities for uncertain tax positions. The Company files income tax returns in the United States, New York State and New York City, but did not report any income effectively connected with a U.S. trade or business. The federal, state and local income tax returns are generally subject to tax examinations for all periods since inception.

10. Commitments and Contingencies

Legal Liabilities

The Company may be subject to various claims and legal proceedings that arise from time to time in the ordinary course of its business. The Company is not involved in any legal proceeding that it expects to have a material effect on its business, financial position, results of operations or cash flows.

11. Agreements Related to Intellectual Property and Manufacturing Agreements

The Company has entered into various license and research and collaboration arrangements. The transactions principally resulted in the acquisition of intellectual property which is in the pre-clinical phase and have not been tested for safety or feasibility. In all cases, the Company did not acquire tangible assets, processes, protocols or operating systems. The Company expenses the acquired intellectual property assets as of the acquisition date on the basis that the cost of intangible assets purchased from others for use in research and development activities, has no alternative future uses.

License 161101 and SRA 161101

On November 17, 2015, the Company entered into an exclusive license agreement ("License 161101") with Fred Hutchinson Research Cancer Center ("Hutch") under which the Company was granted an exclusive license under the patents specified in the agreement of the License 161101 (the "Patents") to make, have made, use, sell, offer to sell, and import products and processes using gene therapy for the treatment of FA. The Company additionally has the right to grant one or more sublicenses to any or all of the rights licensed in connection with License 161101. The License Agreement is in effect for the earlier of (a) the expiration date of the last-to-expire patent, on a country-by-country basis, in which a valid claim covers a product in the country in which the product is sold, or (b) 15 years following regulatory approval of the first product. The Company will record as expense any contingent milestone payments or royalties in the period in which such liabilities are incurred.

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The Company is obligated to make aggregate payments of up to \$1,600 to Hutch upon the achievement of specified development and regulatory milestones. With respect to any commercialized products covered by License 161101, the Company is obligated to pay a low to mid-single digit royalty on net sales, subject to specified adjustments, by the Company or its sub licensees or affiliates. In the event that the Company enters into a sublicense agreement with a sub licensee, the Company will be obligated to pay a portion of any consideration the Company receives from such sublicenses in specified circumstances.

The Company may terminate this agreement at any time by providing Hutch with 180 days advance notice. License 161101 was amended on January 16, 2016 to include additional patents. No additional consideration was provided by the Company and no other changes were made to the terms of License 161101.

Concurrent with License 161101, the Company entered into Sponsored Research Agreement SRA161101 (“SRA 161101”) with Hutch, whereby Hutch will perform a research program in accordance with the Research Plan agreed to between the two parties (as defined in SRA 161101). SRA161101 began on November 19, 2015 and continued for one year and was renewed by mutual agreement of the parties.

On March 6, 2017, the Company entered into a clinical trial agreement with Hutch to perform a clinical trial entitled: Gene Therapy for Patients with Fanconi Anemia Complementation Group A. The Company is obligated to make aggregate payments of \$1,223 inclusive of the Company paying \$136 upon signing the agreement. The Company may terminate this agreement at any time by providing Hutch 30 days advance notice.

License 161201 and SRA 161201

On December 23, 2015, the Company entered into an exclusive license agreement (“License 161201”) with Hutch under which the Company was granted an exclusive license under the patents specified in License 161201 (the “Patents”) to make, have made, use, sell, offer to sell, and import products and processes. The Company additionally has the right to grant one or more sublicenses to all of the rights licensed in connection with the License Agreement. The License Agreement is in effect for the earlier of (a) the expiration date of the last-to-expire patent, on a country-by-country basis, in which a valid claim covers a product in the country in which the product is sold, or (b) 15 years following regulatory approval of the first product.

The Company is obligated to make aggregate payments of up to \$200 to Hutch upon the achievement of specified development and regulatory milestones. In the event that the Company enters into a sublicense agreement with a sub licensee, the Company will be obligated to pay a portion of any consideration the Company receives from such sublicenses in specified circumstances. During the year ended December 31, 2017, the Company paid \$50 for the achievement of specified developmental milestones.

The Company may terminate this agreement at any time by providing Hutch with 180 days advance notice.

Concurrent with License 161201, the Company entered into Sponsored Research Agreement SRA161201 (“SRA 161201”) with Hutch, whereby Hutch will perform a research program in accordance with the Research Plan agreed to between the two parties (as defined in SRA 161201). SRA 161201 began on December 23, 2015 and continued for one year and is under negotiation to renew by mutual agreement. The Company and Hutch expect the Research Plan to continue for a five-year period.

The Company is obligated and made payments under the first-year budget for SRA 161201 of \$580. In the event the Research Agreement is terminated early, Hutch agrees to return any unexpended and uncommitted funds. It is anticipated that the total funding provided by the Company to the third party for the Research Agreement and the Investigator-Initiated Study Agreement will be up to \$1,850; however, the details and annual budget will be determined by the mutual agreement of the Company and Hutch.

PKD (pyruvate kinase deficiency) License Agreement with CIEMAT

On March 8, 2016, the Company entered into an exclusive license agreement (“PKD License”) with CIEMAT under which the Company was granted an exclusive license to develop, make, manufacture, use, commercialize, sell, offer, lease, and import products and processes related to pyruvate kinase deficiency (“PKD”). The Company additionally has the right to grant one or more sublicenses to any or all of the rights listed in connection with the PKD License, with CIEMAT’s prior written consent. The PKD License is in effect for a duration for each of the countries defined in the PKD License for as long as a license right exists that covers the licensed product or process in such country, or until the end of any additional legal protection that should be obtained for the license rights in each country.

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The Company is obligated to make aggregate payments of up to €1,350 to CIEMAT upon the achievement of specified development and regulatory milestones. With respect to any commercialized products covered by the PKD License, the Company is obligated to pay a low to mid-single digit royalty on net sales, subject to specified adjustments, by the Company or its sub licensees or affiliates. In the event that the Company enters into a sublicense agreement with a sub licensee, the Company will be obligated to pay a portion of any consideration the Company receives from such sub licensees in specified circumstances.

During the year ending December 31, 2016, the Company paid CIEMAT an initial upfront license fee of €25 (approximately \$29) which was expensed as research and development costs.

The Company may terminate this agreement at any time by providing CIEMAT with 90 days advance notice.

Concurrent with the PKD License, the Company entered into a Research Cooperation Agreement (“RCA”) with CIEMAT, whereby CIEMAT will perform a research program in accordance with the research program agreed to between the two parties (as defined in the RCA). The RCA began on March 8, 2016 and will continue through December 1, 2020 or such later date as may be mutually agreed upon by the Company and CIEMAT.

The Company is obligated under the RCA to make aggregate payments of €4,190. At the end of the research program, any unspent funds previously paid by the Company shall be returned to the Company within 90 days of the termination or expiration of the research program. Payments made under this agreement are considered advance payments that are refundable by CIEMAT in the event of early termination, and therefore these amounts are expensed as research services are performed.

Master Research Agreement with CIEMAT

On July 7, 2016, the Company entered into a master research agreement (“MRA”) with CIEMAT whereby the Company will co-fund different research programs to be conducted by CIEMAT that would be described in specific exhibits to the MRA, providing up to €5,150 in total funding support across the various research programs. The MRA also stipulates the Company will be entitled to acquire certain rights to intellectual property arising out of the various research programs through a separate license agreement. The term of the MRA is from July 7, 2016 through July 7, 2021 or such later date as may be mutually agreed upon by the Company and CIEMAT.

FA License Agreement with CIEMAT

On July 15, 2016, the Company entered into an exclusive license agreement (“FA License”) with CIEMAT under which the Company was granted an exclusive license to develop, make, manufacture, use, commercialize, sell, offer, lease, and import products and processes related to FA. The Company additionally has the right to grant one or more sublicenses to any or all of the rights listed in connection with the FA License, with CIEMAT’s prior written consent. The FA License is in effect for a duration for each of the countries defined in the FA License for as long as a license right exists that covers the licensed product or process in such country, or until the end of any additional legal protection that should be obtained for the license rights in each country.

The Company is obligated to make aggregate payments of up to €5,025 to CIEMAT upon the achievement of specified development and regulatory milestones. With respect to any commercialized products covered by the FA License, the Company is obligated to pay a mid-single digit royalty on net sales, subject to specified adjustments, by the Company or its sub licensees or affiliates. In the event that the Company enters into a sublicense agreement with a sub licensee, the Company will be obligated to pay a portion of any consideration the Company receives from such sub licensees in specified circumstances.

During the year ending December 31, 2016, Rocket Pharma paid CIEMAT an initial upfront license fee of €125 (approximately \$137) which was expensed as research and development costs.

The Company may terminate this agreement at any time by providing CIEMAT with 90 days advance notice.

LAD-I (leukocyte adhesion deficiency-I) Agreement with CIEMAT

On July 15, 2016, the Company entered into a collaboration agreement (as Annex 2 to the MRA) with CIEMAT to perform research in the area of Lentiviral-mediated gene therapy (the “LAD-I Agreement”) for a period of five years from the effective date or later if mutually agreed.

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The LAD-I Agreement also provides the Company an option to acquire the related intellectual property rights from CIEMAT to make, manufacture, use, commercialize, sell, offer, lease, and import related products and processes. The Company additionally has the right to grant one or more sublicenses to any or all of the rights listed, with CIEMAT's prior written consent. Such future license would be in effect for a duration for each of the countries defined in the LAD-I Agreement for as long as a license right exists that covers the licensed product or process in such country, or until the end of any additional legal protection that should be obtained for the license rights in each country.

The Company is obligated to make aggregate payments of up to €3,040 to CIEMAT upon the achievement of specified development and regulatory milestones in addition to €25 per licensed indication. With respect to any commercialized products covered by a future license agreement, the Company is obligated to pay a low to mid-single digit royalty on net sales, subject to specified adjustments, by the Company or its sub licensees or affiliates. In the event that the Company enters into a sublicense agreement with a sub licensee, the Company will be obligated to pay a portion of any consideration the Company receives from such sub licensees in specified circumstances.

The Company may terminate this agreement at any time by providing CIEMAT 180 days advance notice.

At the end of the research program, any unspent funds previously paid by the Company shall be returned to the Company within 90 days of the termination or expiration of the research program. Payments under this agreement are considered advance payments that are refundable by CIEMAT in the event of early termination, and therefore these amounts will be expensed as service is performed over the term of the agreement.

License Agreement for LAD-I with CIEMAT and UCLB

Rocket Pharma entered into a license agreement in November 2017, effective September 2017, with CIEMAT, CIBER, and FIISFJD (which we refer to collectively as CIEMAT) and UCL Business PLC ("UCLB"), collectively referred to as Licensors, granting Rocket worldwide, exclusive rights to certain patents. As consideration for the licensed rights, Rocket will pay the Licensors an initial license fee of €25 (approximately \$30). Rocket Pharma is obligated to make aggregate payments of up to €1,350 (approximately \$1,600) to Licensors upon the achievement of specified development and regulatory milestones. Rocket Pharma may terminate this agreement at any time by providing the Licensors 90 days advance notice. During 2017, the Company paid a portion of the license fee and accrued the balance of \$26 of the license fee as of December 31, 2017.

Research and Collaboration Agreement with Lund University and Researcher

On August 16, 2016, The Company entered into a contract research and collaboration agreement with Lund University ("LU") and a researcher (collectively "the LU Agreement") to perform gene therapy research in the area of Infantile Malignant Osteopetrosis for a period of two years from the effective date or completion of the activities, whichever is the earliest.

The Agreement provides the Company an option to acquire rights from LU in the following manner. Either a) acquire ownership rights to the results on commercially reasonable terms and conditions or b) acquire, on commercially reasonable terms and conditions, an exclusive or non-exclusive, perpetual, assignable license, which may be regionally or field limited, with the right to sub-license, to make, have made, use, import, offer to sell and sell the results under any and all rights which LU has to results. Additionally, the Agreement provides the Company an option to acquire the Researcher's ownership rights to the results on commercially reasonable terms and conditions.

The Company is obligated to make aggregate payments of up to €478 to LU and the Researcher upon the achievement of specified development and regulatory milestones. With respect to any commercialized products covered by the LU Agreement, the Company is obligated to pay a low single digit royalty on net sales, subject to specified adjustments, by the Company or its sub licensees or affiliates. In the event that the Company enters into a sublicense agreement with a sub licensee, the Company will be obligated to pay a portion of any consideration the Company receives from such sub licensees in specified circumstances.

During the year ending December 31, 2016, the Company paid Lund an upfront license fee of €20 (approximately \$24) which was expensed as research and development costs.

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The Company may terminate this agreement at any time by providing LU and the Researcher with 90 days' advance notice.

Payments under this agreement are considered advance payments that are refundable by LU and the Researcher in the event of early termination, and therefore these amounts are expensed as service is performed over the term of the agreement.

As of December 31, 2017, the Company had total remaining commitments under these agreements of \$1,331.

Manufacturing Agreements

During the year ended December 31, 2017, the Company entered into non-cancelable commitments with multiple contract manufacturers. Total remaining commitments under these contracts were \$1,296 at December 31, 2017.

12. Related Party Transaction

The Company is party to a consulting agreement with a sibling of one of the Company's key employees to provide professional real estate advisory services. During the year ended December 31, 2017, the Company incurred expenses in connection with this agreement totaling \$37. The agreement may be terminated by the Company upon 14 days written notice.

The Company received an advance of \$14 from an officer of the Company in 2015 which was repaid in January 2016.

13. Operating Lease

On March 31, 2016, the Company entered into a lease agreement for its office space which has a term ending on July 31, 2021. Rent expense associated with this operating lease for the years ended December 31, 2017 and 2016 was \$427 and \$358. Minimum lease payments due under the lease for subsequent years are summarized in the table below:

2018	440
2019	453
2020	467
2021	278
Total minimum lease payments	<u>\$1,638</u>

In connection with the lease agreement, the Company established an irrevocable standby letter of credit ("LOC") with a bank. The LOC's serves as the Company's security deposit on the lease, with the landlord as the beneficiary. The LOC expires and is automatically renewed April 8 of each succeeding calendar year up to October 29, 2021, unless written notice is provided no later than 90 days before the then existing expiration date. The Company has restricted cash to the bank as collateral for the stand by letter of credit. The restricted cash balance at December 31, 2017 and 2016 was \$207 and \$205.

14. 401(k) Savings Plan

Effective January 1, 2016, the Company has a defined contribution savings plan (the "Plan") under Section 401(k) of the Internal Revenue Code. This Plan covers substantially all employees who meet minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pre-tax basis. Company contributions to the Plan may be made at the discretion of the Company's board of directors. The Company has elected to match 4% of employee contributions to the Plan, subject to certain limitations. The Company's matching contribution for the years ended December 31, 2017 and 2016, was \$101 and \$28, respectively.

15. Subsequent Events

On January 24, 2018, Rocket entered into an underwriting agreement (the "Underwriting Agreement") with Cowen and Company, LLC and Evercore Group L.L.C., as representatives (the "Representatives") of the several underwriters (collectively with the Representatives, the "Underwriters"), pursuant to which Rocket sold 6,325,000 shares of common stock (the "Shares"), which includes 825,000 shares that were sold pursuant to an option granted to the Underwriters (the "Offering"). The Shares were sold in the Offering at a public offering price of \$13.25 per share in which Rocket received net proceeds from the Offering of \$78.8 million, after deducting the underwriting discounts and commissions.

The Company has evaluated events and transactions subsequent to the balance sheet date through the time these financial statements were available for issuance on March 7, 2018.