

**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): February 16, 2018**

**Spark Therapeutics, Inc.**  
(Exact Name of Registrant as Specified in its Charter)

**Delaware**  
(State or Other Jurisdiction  
of Incorporation)

**001-36819**  
(Commission  
File Number)

**46-2654405**  
(IRS Employer  
Identification No.)

**3737 Market Street  
Suite 1300  
Philadelphia, PA**  
(Address of Principal Executive Offices)

**19104**  
(Zip Code)

**Registrant's telephone number, including area code: (888) 772-7560**

**(Former Name or Former Address, if Changed Since Last Report)**

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (*see* General Instruction 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))
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**Item 1.01. Entry into a Material Definitive Agreement.**

On February 16, 2018, Spark Therapeutics, Inc. (the “Company”) entered into a SPK-9001 Manufacture and Supply Agreement (the “Agreement”) with Pfizer Inc. (“Pfizer”) to manufacture and deliver to Pfizer SPARK-9001 bulk drug substance (the “Product”).

Under the Agreement, Pfizer will pay the Company \$7.0 million upon execution of the Agreement and may make a second payment to the Company of up to \$7.0 million based on (i) the date by which the Company provides Pfizer analytical results of the Product deliverable and (ii) the volume of Product delivered.

The Agreement continues until 30 days after the Company has supplied one batch of Product to Pfizer. The Company will begin Product manufacture in the first quarter of 2018.

Either party may terminate the Agreement upon the other party’s uncured material breach of the Agreement, insolvency, or bankruptcy. Pfizer also may terminate the Agreement in the event of the Company’s breach of the anti-bribery/anti-corruption representation or the global trade representation.

The foregoing descriptions of the Agreement do not purport to be complete and are subject to, and qualified in their entirety by reference to, the full text of the Agreement. The Company intends to file a copy of the Agreement with the Company’s Quarterly Report on Form 10-Q for the quarter ended March 31, 2018.

**Item 2.02. Results of Operations and Financial Condition.**

On February 20, 2018, the Company issued a press release announcing unaudited consolidated financial results for the year ended December 31, 2017. A copy of the press release is being filed as Exhibit 99.1 to this Current Report on Form 8-K.

The information in this Form 8-K (including Exhibit 99.1) 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 preference in any filing under the Securities Act of 1933 or the Exchange Act, except as expressly set forth by specific reference in such a filing.

**Item 9.01. Financial Statements and Exhibits.**

(d) Exhibits

The following exhibit relating to Item 2.02 shall be deemed to be furnished, and not filed:

[Exhibit 99.1](#) [Press release issued by Spark Therapeutics, Inc., dated February 20, 2018.](#)

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SIGNATURES

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.

SPARK THERAPEUTICS, INC.

Date: February 20, 2018

By: /s/ Joseph W. La Barge  
Joseph W. La Barge  
General Counsel

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**Exhibit Index**

[Exhibit 99.1](#)

[Press release issued by Spark Therapeutics, Inc., dated February 20, 2018](#)

## Spark Therapeutics Reports 2017 Financial Results and Recent Business Progress

*U.S. Food and Drug Administration (FDA) grants breakthrough therapy designation to SPK-8011 for hemophilia A*

**PHILADELPHIA, Feb. 20, 2018** (GLOBE NEWSWIRE)- Spark Therapeutics (NASDAQ: ONCE), a fully integrated gene therapy company dedicated to challenging the inevitability of genetic disease, announced today corporate and financial results for 2017 and recent business progress.

“The landmark approval of LUXTURNA™ (voretigene neparvovec-rzyl) in December as the first gene therapy for a genetic disease in the U.S. topped another year of great progress for Spark Therapeutics,” said Jeffrey D. Marrazzo, chief executive officer of Spark Therapeutics. “We have built a fully integrated company dedicated to discovering, developing and delivering one-time treatments that provide long-lasting, transformative outcomes to patients, families, society and the health care system. In 2018, we are focused on successfully launching LUXTURNA in the U.S. and securing marketing authorization in the EU, advancing our global development program for *SPK-8011* in hemophilia A and continuing to progress our pipeline of other investigational gene therapies.

“Additionally, FDA recently has granted breakthrough therapy designation to *SPK-8011* for hemophilia A. It marks the third time we have been granted this designation for as many investigational gene therapies, a signal of the strength of our expertise in gene therapy,” adds Marrazzo.

### 12-month highlights

*Received FDA approval for LUXTURNA, a one-time gene therapy product indicated for the treatment of patients with confirmed biallelic RPE65 mutation-associated retinal dystrophy who have viable retinal cells as determined by their treating physicians:*

- Achieved product labeling for LUXTURNA that provides a genetically based indication with a clearly described safety profile from the clinical development program
- Announced novel payer and patient offerings to help ensure that appropriate patients have access to LUXTURNA
- First and only FDA-approved adeno-associated virus (AAV) product in the U.S.
- First and only FDA-approved AAV commercial manufacturing facility

*Entered into licensing and supply agreement granting Novartis Pharmaceuticals exclusive rights to commercialize voretigene neparvovec in markets outside the U.S.*

- Received \$105 million up front in January 2018
  - Eligible to receive an additional \$25 million upon approval by European Medicines Agency (EMA) and total of \$40 million in aggregate additional milestones on initial sales in multiple ex-U.S. markets
  - Receive a flat, mid-20 percent royalty on annual net sales outside the U.S.
  - Spark Therapeutics retains exclusive commercial rights to LUXTURNA in the U.S.
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*Advanced two investigational hemophilia programs with no reported serious adverse events, thrombotic events or inhibitors to date and clinically meaningful reductions in annualized bleeding rate (ABR) and annualized infusion rate (AIR)*

- Showed predictable clinical outcomes one-year post-infusion of investigational *SPK-9001* in hemophilia B without introducing new or unforeseen risks
  - Published interim Phase 1/2 clinical trial data of *SPK-9001* in *The New England Journal of Medicine*
  - At American Society of Hematology (ASH), released more than 13 years of cumulative follow-up data on participants in the *SPK-9001* Phase 1/2 trial demonstrating a 97-percent reduction in ABR and a 99-percent reduction in AIR calculated based on data after week four, as of the Nov. 29, 2017 data cutoff
  - Entered into an amendment to the license agreement for *SPK-9001* with Pfizer, Inc., in November 2017; including an initial \$10 million cash payment and up to an additional \$15 million in potential milestone payments upon completion of certain transitional activities in mid-2018
  - Entered into a supply agreement with Pfizer in February 2018 to begin production this quarter for one batch of drug substance expected to be used for Phase 3 development; Spark received \$7 million up front and will receive up to \$7 million upon delivery
- Demonstrated initial human proof-of-concept for investigational *SPK-8011* in hemophilia A
  - Presented early *SPK-8011* Phase 1/2 clinical trial data at ASH for the first four participants who had been followed at least 12 weeks post infusion as of the Dec. 6, 2017 data cutoff
    - Reported a 100-percent reduction in ABR and 98-percent reduction in AIR calculated based on data after week four
  - FDA granted orphan-disease designation to *SPK-8011* in January 2018
  - FDA granted breakthrough therapy designation to *SPK-8011* in February 2018

*Progressed pipeline of investigational gene therapies*

- Completed enrollment of five earlier-stage choroideremia participants in Phase 1/2 clinical trial for *SPK-7001*
- Licensed a liver-directed, AAV gene therapy candidate from Genethon for Pompe disease

*Bolstered human capital, technology platform and financial position:*

- Added Robert J. Perez, a long-time biopharmaceutical executive, to the board of directors
  - Received a rare pediatric disease priority review voucher in conjunction with the approval of LUXTURNA
  - Strong balance sheet with \$540.2 million in cash, cash equivalents and marketable securities as of Dec. 31, 2017, excluding the \$105 million received from Novartis in January 2018
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### **Financial results for the year ended Dec. 31, 2017 and 2016**

In the year ended Dec. 31, 2017, we recognized \$12.1 million in revenue, all of which was associated with our Pfizer agreement. In the year ended Dec. 31, 2016, we recognized \$20.2 million in revenue, which was all associated with our Pfizer agreement, and included a \$15.0 million milestone payment that was earned in December 2016.

Research and development expenses for the year ended Dec. 31, 2017 were \$135.2 million versus \$86.4 million for the year ended Dec. 31, 2016. The \$48.8 million increase was due to a \$40.4 million increase in internal research and development expenses, due to increased effort and headcount in research, technical operations, diagnostics, quality assurance and quality control and an increase of \$8.4 million in external research and development expenses, primarily driven by a \$4.4 million increase in expenses related to our hemophilia A program.

Our acquired in-process research and development expense for the year ended Dec. 31, 2017 was \$8.6 million, which includes additional payments related to our Selecta Bioscience, Inc. (Selecta) license agreement entered into in 2016. Our acquired in-process research and development (IPR&D) expense for the year ended Dec. 31, 2016 was \$11.1 million. This amount represents the upfront payment related to the Selecta license agreement.

During the year ended Dec. 31, 2017, we recorded a non-cash impairment charge of \$15.7 million related to acquired IPR&D from a March 2016 acquisition. Additionally, we recognized an income tax benefit of \$1.0 million related to the reversal of the deferred tax liability associated with the IPR&D during the year ended Dec. 31, 2017.

General and administrative expenses for the year ended Dec. 31, 2017 were \$111.1 million versus \$48.1 million for the year ended Dec. 31, 2016. General and administrative expenses consist primarily of salaries and related costs, including stock-based compensation, legal and patent costs and other professional fees. The \$63.0 million increase primarily was due to an increase of \$27.8 million in salaries and related costs, including stock-based compensation linked to increased headcount, an increase of \$11.9 million in launch preparation activities for LUXTURNA, \$14.7 million in legal and patent expenses, professional fees and other operating costs, and \$8.6 million in facility related costs.

Our net loss for the year ended Dec. 31, 2017 was \$253.5 million, or (\$7.63) basic and diluted net loss per common share, as compared with a net loss of \$123.7 million, or (\$4.29) basic and diluted net loss per common share for the year ended Dec. 31, 2016.

As of Dec. 31, 2017, Spark had cash and cash equivalents and marketable securities of \$540.2 million.

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### Conference call details

Spark Therapeutics will host a conference call and audio webcast, today, Tuesday, Feb. 20, at 8:30 a.m. ET, to discuss corporate and financial results for 2017 and recent business highlights. The call can be accessed by dialing the numbers below or by visiting the "Investors" section at [www.sparktx.com](http://www.sparktx.com).

U.S. Dial-in Number: (855) 851-4526

International Dial-in Number: (720) 634-2901

Passcode: 9596288

A replay of the call will be available for one week following the call by dialing the numbers below or also available on our website.

Replay Dial-in Number: (855) 859-2056

Replay International Dial-in Number: (404) 537-3406

Passcode: 9596288

### About Spark Therapeutics

At Spark Therapeutics, a fully integrated company committed to discovering, developing and delivering gene therapies, we challenge the inevitability of genetic diseases, including blindness, hemophilia and neurodegenerative diseases. We have successfully applied our technology in the first FDA-approved gene therapy in the U.S. for a genetic disease, and currently have three programs in clinical trials, including product candidates that have shown promising early results in patients with hemophilia. At Spark, we see the path to a world where no life is limited by genetic disease. For more information, visit [www.sparktx.com](http://www.sparktx.com), and follow us on [Twitter](#) and [LinkedIn](#).

### Cautionary note on forward-looking statements

This release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding the company's product candidates, including LUXTURNA, *SPK-7001*, *SPK-9001* and *SPK-8011*. The words "anticipate," "believe," "expect," "intend," "may," "plan," "predict," "will," "would," "could," "should," "continue" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Any forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in, or implied by, such forward-looking statements. These risks and uncertainties include, but are not limited to, the risk that (i) we may not successfully launch LUXTURNA in the U.S., or our success may be delayed; (ii) our MAA for LUXTURNA may not be approved by EMA; (iii) the data from our Phase 3 clinical trial of LUXTURNA may not support labeling for all biallelic *RPE65* mutations other than Leber congenital amaurosis (LCA) in ex-US geographies; (iv) the improvements in functional vision demonstrated by LUXTURNA in our clinical trials may not be sustained over extended periods of time; (v) voretigene neparvovec may not be approved in any markets outside of the U.S.; (vi) upon approval, Novartis may not be successful in commercializing or selling voretigene neparvovec in one or more markets; (vii) we may not receive any additional milestone or royalty payments from Novartis, Pfizer, or our other collaborators; (viii) our early preliminary clinical results for our product candidate, *SPK-8011*, for hemophilia A may not be sustained or sufficient to support further development; (ix) we may be unsuccessful in achieving higher factor VIII activity levels through dose escalation in our Phase 1/2 clinical trial of *SPK-8011*; (x) we are unable to enter into agreements with payers for the provision of LUXTURNA; (xi) we will not be able to reach agreement with the Centers for Medicare & Medicaid Services (CMS) regarding LUXTURNA; (xii) our lead *SPK-FIX* product candidate, *SPK-9001*, may not produce sufficient data in our Phase 1/2 clinical trial to warrant further development; (xiii) interim data from our *SPK-7001* Phase 1/2 clinical trial, including data to be generated from our recently expanded cohort, may not support further development of this product candidate; and (xiv) any one or more of our product candidates in preclinical or clinical development will not successfully be developed and commercialized. For a discussion of other risks and uncertainties, and other important factors, any of which could cause our actual results to differ from those contained in the forward-looking statements, see the "Risk Factors"

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section, as well as discussions of potential risks, uncertainties and other important factors, in our Annual Report on Form 10-K, our Quarterly Reports on Form 10-Q and other filings we make with the Securities and Exchange Commission. All information in this press release is as of the date of the release, and Spark Therapeutics undertakes no duty to update this information unless required by law.

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**Spark Therapeutics, Inc.**  
**Consolidated balance sheets**  
**(unaudited)**

	December 31, 2016	December 31, 2017
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 58,923,097	\$ 96,748,444
Marketable securities	237,242,655	423,418,752
Other receivables	16,780,917	7,905,653
Prepaid expenses	1,647,008	5,092,877
Total current assets	314,593,677	533,165,726
Marketable securities	21,900,129	20,035,553
Property and equipment, net	19,794,306	61,712,793
Acquired in-process research and development	15,490,000	—
Goodwill	1,160,104	1,254,005
Other assets	924,579	628,235
Total assets	<u>\$ 373,862,795</u>	<u>\$ 616,796,312</u>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 9,928,737	\$ 14,182,804
Accrued expenses	13,826,920	24,697,225
Current portion of long-term debt	302,013	311,976
Current portion of deferred rent	771,196	968,534
Current portion of deferred revenue	5,168,674	11,968,915
Current other liabilities	—	1,557,062
Total current liabilities	29,997,540	53,686,516
Long-term debt	1,224,003	912,027
Long-term deferred rent	7,498,419	8,317,952
Long-term deferred revenue	3,865,885	—
Deferred tax liability	1,000,235	—
Other liabilities	—	40,255,605
Total liabilities	43,586,082	103,172,100
Stockholders' equity:		
Preferred stock, \$0.001 par value. Authorized, 5,000,000 shares; no shares issued or outstanding	—	—
Common stock, \$0.001 par value. Authorized, 150,000,000 shares; 30,873,430 shares issued and 30,864,224 shares outstanding as of December 31, 2016; 37,131,626 shares issued and 37,111,404 shares outstanding as of December 31, 2017	30,874	37,132
Additional paid-in capital	583,973,682	1,026,589,507
Accumulated other comprehensive loss	(794,296)	(5,913,595)
Treasury stock, at cost, 9,206 shares as of December 31, 2016 and 20,222 shares as of December 31, 2017	(552,636)	(1,225,949)
Accumulated deficit	(252,380,911)	(505,862,883)
Total stockholders' equity	330,276,713	513,624,212
Total liabilities and stockholders' equity	<u>\$ 373,862,795</u>	<u>\$ 616,796,312</u>

**Spark Therapeutics, Inc.**  
**Consolidated statements of operations**  
**(unaudited)**

	<b>For the Year Ended December 31,</b>		
	<b>2015</b>	<b>2016</b>	<b>2017</b>
Revenues	\$ 22,063,674	\$ 20,182,835	\$ 12,065,644
Operating expenses:			
Research and development	46,029,314	86,379,405	135,160,047
Acquired in-process research and development	—	11,132,146	8,604,258
Impairment of in-process research and development	—	—	15,696,017
General and administrative	23,352,171	48,070,317	111,123,247
Total operating expenses	<u>69,381,485</u>	<u>145,581,868</u>	<u>270,583,569</u>
Loss from operations	(47,317,811)	(125,399,033)	(258,517,925)
Interest income, net	<u>192,033</u>	<u>1,746,506</u>	<u>4,072,912</u>
Loss before income taxes	(47,125,778)	(123,652,527)	(254,445,013)
Income tax benefit	<u>—</u>	<u>—</u>	<u>963,041</u>
Net loss	(47,125,778)	(123,652,527)	(253,481,972)
Preferred stock dividends	(634,794)	—	—
Net loss applicable to common stockholders	<u>\$ (47,760,572)</u>	<u>\$ (123,652,527)</u>	<u>\$ (253,481,972)</u>
Basic and diluted net loss per common share	<u>\$ (2.10)</u>	<u>\$ (4.29)</u>	<u>\$ (7.63)</u>
Weighted average basic and diluted common shares outstanding	<u>22,710,105</u>	<u>28,804,133</u>	<u>33,242,072</u>

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