# 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): August 2, 2017**

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

| **Delaware** | **001-36819** | **46-2654405** |
|---|---|---|
| **(State or Other Jurisdiction of Incorporation)** | **(Commission File Number)** | **(IRS Employer Identification No.)** |

| **3737 Market Street<br>Suite 1300<br>Philadelphia, PA** | **19104** |
|---|---|
| **(Address of Principal Executive Offices)** | **(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))

Indicate by check mark whether the registrant is an emerging growth company as defined in 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 Operations and Financial Condition**

On August 2, 2017, Spark Therapeutics, Inc. issued a press release announcing unaudited consolidated financial results for the quarter ended June 30, 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 Current Report on 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 reference 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 August 2, 2017. |

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: August 2, 2017

By: /s/ Joseph W. La Barge

Joseph W. La Barge

Chief Legal Officer

**Exhibit Index**

| | |
|---|---|
| Exhibit 99.1 | Press release issued by Spark Therapeutics, Inc., dated August 2, 2017. |

**Exhibit 99.1**

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

*Preliminary interim data for* SPK-8011 *Phase 1/2 dose-escalation clinical trial in hemophilia A show initial human proof-of-concept in three participants*

*In the two participants at the initial dose, stable factor VIII levels average greater than 12 percent with no spontaneous bleeds*

*Investigational LUXTURNA™ (voretigene neparvovec) Prescription Drug User Fee Act (PDUFA) date set for Jan. 12, 2018; granted rare pediatric disease designation by FDA*

**PHILADELPHIA, Aug. 2, 2017** (GLOBE NEWSWIRE)- Spark Therapeutics (NASDAQ: ONCE), a fully integrated gene therapy company dedicated to challenging the inevitability of genetic disease, announced today financial results for the second quarter of 2017 and recent business progress.

"This has been an unprecedented time at Spark Therapeutics, marked by the achievement of significant regulatory milestones for investigational LUXTURNA™ (voretigene neparvovec). The U.S. Food and Drug Administration (FDA) has accepted for filing the Biologics License Application (BLA) for LUXTURNA, which is the first BLA for a gene therapy for an inherited disease, and we have submitted the marketing authorization application (MAA) to the European Medicines Agency (EMA). Additionally, LUXTURNA has been designated by FDA as a drug for a rare pediatric disease, which will qualify us for a Priority Review Voucher, if LUXTURNA is approved," said Jeffrey D. Marrazzo, chief executive officer of Spark Therapeutics. "These critical steps all get us closer to potentially bringing the first treatment to patients with vision loss due to confirmed biallelic *RPE65*-mediated inherited retinal disease (IRD). A natural history study has shown that people with this IRD eventually progress to complete blindness."

The company also disclosed preliminary initial data from the Phase 1/2 dose-escalation clinical trial for *SPK-8011,* a novel bio-engineered adeno-associated viral (AAV) vector utilizing the Spark200 capsid and containing a codon-optimized human factor VIII gene, being investigated as a potential one-time therapy for hemophilia A. It is the second investigational hemophilia gene therapy to emerge from Spark Therapeutics' leading gene therapy platform. Spark Therapeutics is developing *SPK-8011* independently and retains global commercialization rights to the *SPK-FVIII* program.

"The encouraging start of our *SPK-8011* clinical trial reinforces the strength of our gene therapy platform, delivers human proof-of-concept in a second liver-mediated disease -- a significant achievement in the gene therapy field -- and positions us well to potentially transform the current treatment approach for this life-altering disease with a one-time intervention," said Katherine A. High, president and chief scientific officer of Spark Therapeutics. "We are

excited about the progress we are making to achieve our goals of our investigational hemophilia A and B programs: to safely achieve predictable, consistent and sustained activity levels that prevent spontaneous bleeding."

With three participants infused in the hemophilia A dose-escalation clinical trial, there have been no serious adverse events (SAEs) reported to date, including no factor VIII inhibitors and no thrombotic events. As of the Aug. 1, 2017 data cutoff, the first two participants, who have received a single administration of *SPK-8011* at an initial dose of 5 x $10^{11}$ vector genomes (vg)/kg body weight, have now been followed for 23 weeks and 12 weeks, respectively. During this time, there has been a steady and consistent rise in factor VIII activity levels that have now stabilized at levels of 11 percent and 14 percent of normal, respectively.

Based on these data, the company elected to double the dose and recently infused a third participant at a dose of 1 x $10^{12}$ vector genomes (vg)/kg body weight. While the results for this third participant are early, his factor activity level is tracking proportionally higher, consistent with the dose escalation.

At this point in the trial, there have been no immune responses and none of the participants has required use of corticosteroids. There have been no reported spontaneous bleeds.

**Additional highlights**

*Advanced investigational LUXTURNA for the treatment of biallelic* RPE65*-mediated IRD*:

- Progressed regulatory submissions in the U.S. and EU
    - BLA accepted with target PDUFA date set for Jan. 12, 2018
    - Received rare pediatric disease designation for vision loss due to confirmed biallelic *RPE65* mutation-associated retinal dystrophy from FDA
    - Submitted MAA to EMA on July 29, 2017
- Published Phase 3 trial clinical trial data for LUXTURNA in *The Lancet*, including the intent-to-treat population of all randomized participants, through the one-year timepoint

*Reported updated data from investigational* SPK-9001 *for hemophilia B Phase 1/2 clinical trial at International Society on Thrombosis and Haemostasis (ISTH) 2017 Congress:*

- No SAEs have been reported to date, including no factor IX inhibitors and no thrombotic events
- Since vector administration, as of the June 5, 2017 data cutoff, among the 10 participants, the annualized infusion rate (AIR) had been reduced by 99 percent and the annual bleeding rate (ABR) was reduced by 96 percent
    - Mean steady-state factor IX activity level post-12 weeks treatment for 10 participants was a consistent and sustained 33 percent

- Data represent approximately 10 cumulative patient years of *SPK-9001* exposure from the start of the trial, with one participant out approximately 18 months post-infusion and four additional participants at least one year post-infusion
    - Only one participant, with severe joint disease, has self-administered precautionary infusions for persistent knee pain
- Two participants who had experienced an asymptomatic, transient elevation in liver enzymes or a decline in factor IX activity have continued to demonstrate stable factor IX activity levels, 18 and 12 weeks' post-cessation of steroids, respectively.
    - Neither of these participants has experienced a bleed nor taken factor concentrates.

**Financial results for the quarter ended June 30, 2017**

*Three Months Ended June 30, 2017 and 2016*

In the three months ended June 30, 2017 and 2016, we recognized $1.5 million and $1.3 million, respectively, of revenue associated with our Pfizer collaboration.

Our research and development expenses for the three months ended June 30, 2017 were $33.0 million versus $19.6 million for the three months ended June 30, 2016. The $13.4 million increase was due to a $9.8 million increase in internal research and development expenses, primarily due to increased headcount, and an increase of $3.6 million in external research and development. The increase in external research and development was primarily from an increase of $1.7 million in expenses related to LUXTURNA, $1.2 million in our other programs in preclinical development and $0.7 million in our other clinical programs.

Our acquired in-process research and development expense for the three months ended June 30, 2017 was $3.1 million versus zero for the three months ended June 30, 2016. This amount represents a payment related to our license agreement with Selecta Biosciences, Inc., or Selecta.

During the three months ended June 30, 2017, we recorded a non-cash impairment charge of $15.7 million related to acquired in-process research and development, or IPR&D, attained in March 2016. 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 three months ended June 30, 2017.

General and administrative expenses for the three months ended June 30, 2017 were $26.7 million versus $10.7 million for the three months ended June 30, 2016. General and administrative expenses consisted primarily of salaries and related costs, including stock-based compensation, legal and patent costs, professional fees and other operating costs. The $16.1 million increase primarily was due to an increase of $7.2 million in salaries and related costs, including stock-based compensation, as a result of increased headcount, an increase of $4.0 million in launch preparation activities for LUXTURNA and $4.9 million in legal and patent costs, professional fees and other operating costs.

During the three months ended June 30, 2017, we recorded an income tax benefit of $1.1 million related to our available for sale securities.

Our net loss for the three months ended June 30, 2017 was $74.4 million, or ($2.40) basic and diluted net loss per common share, as compared with a net loss of $28.7 million, or ($1.04) basic and diluted net loss per common share for the three months ended June 30, 2016.

*Six Months Ended June 30, 2017 and 2016*

In the six months ended June 30, 2017 and 2016, we recognized $2.8 million and $2.6 million, respectively, of revenue associated with our Pfizer collaboration.

Our research and development expenses for the six months ended June 30, 2017 were $65.3 million versus $37.9 million for the six months ended June 30, 2016. The $27.5 million increase was due to a $20.8 million increase in internal research and development expenses, primarily due to increased headcount, and an increase of $6.7 million in external research and development. The increase in external research and development was primarily from an increase of $3.1 million in expenses related to LUXTURNA, $1.8 million in our other programs in preclinical development and $1.8 million in our other clinical programs.

Our acquired in-process research and development expense for the six months ended June 30, 2017 was $3.5 million versus zero for the three months ended June 30, 2016. This amount represents an expense related to our license agreement with Selecta.

During the six months ended June 30, 2017, we recorded a non-cash impairment charge of $15.7 million related to acquired IPR&D attained in March 2016. 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 three months ended June 30, 2017.

General and administrative expenses for the six months ended June 30, 2017 were $48.1 million versus $19.6 million for the six months ended June 30, 2016. General and administrative expenses consisted primarily of salaries and related costs, including stock-based compensation, legal and patent costs, professional fees and other operating costs. The $28.6 million increase primarily was due to an increase of $13.5 million in salaries and related cost, including stock-based compensation, as a result of increased headcount, an increase of $5.8 million in launch preparation activities for LUXTURNA and $9.3 million in legal and patent costs, professional fees and other operating costs.

During the six months ended June 30, 2017, we recorded an income tax benefit of $1.1 million related to our available for sale securities.

Our net loss for the six months ended June 30, 2017 was $126.6 million, or ($4.10) basic and diluted net loss per common share, as compared with a net loss of $54.3 million, or ($2.00) basic and diluted net loss per common share for the six months ended June 30, 2016.

As of June 30, 2017, we had cash and cash equivalents and marketable securities of $238.6 million, with 31.2 million shares outstanding.

**Conference call details**
Spark Therapeutics will host a conference call and audio webcast, today, Wednesday, Aug. 2, at 8:30 a.m. ET, to discuss financial results for the second quarter of 2017 and recent business progress. The call can be accessed by dialing the numbers below or by visiting the "Investors" section at www.sparktx.com.

U.S. Dial-in Number: (855) 851-4526
International Dial-in Number: (720) 634-2901
Passcode: 62580535

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: 62580535

**About Spark Therapeutics**
Spark Therapeutics, a fully integrated company, strives to challenge the inevitability of genetic disease by discovering, developing, and delivering gene therapies that address inherited retinal diseases (IRDs), neurodegenerative diseases, as well as diseases that can be addressed by targeting the liver, such as hemophilia. Spark Therapeutics has ongoing clinical trials investigating gene therapies in hemophilia A and B. *SPK-8011* is in an ongoing, dose-escalation Phase 1/2 clinical trial as a potential one-time therapy for hemophilia A. The company retains full global commercialization rights to the *SPK-FVIII* program. *SPK-9001*, which has received both breakthrough therapy and orphan product designations by FDA, and access to the PRIority MEdicines (PRIME) Program by EMA, is in a Phase 1/2 clinical trial for hemophilia B and is being developed in collaboration with Pfizer. Our most advanced investigational candidate, with proposed trade name LUXTURNA™ (voretigene neparvovec), is currently under Priority Review with FDA for the treatment of biallelic *RPE65*-mediated IRD and has been designated as a drug for a rare pediatric disease. The MAA for LUXTURNA has been submitted to EMA for the treatment of vision loss due to Leber congenital amaurosis or retinitis pigmentosa caused by confirmed biallelic *RPE65* mutations. LUXTURNA has received breakthrough therapy and orphan product designations from FDA and

orphan product designations from EMA. The pipeline also includes *SPK-7001* in an ongoing Phase 1/2 clinical trial for choroideremia. For more information on our pipeline, visit www.sparktx.com.

**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 (voretigene neparvovec), *SPK-7001, SPK-9001* and *SPK-8011*. 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) our BLA or MAA for LUXTURNA may not be approved by the FDA or EMA respectively; (ii) 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); (iii) the improvements in functional vision demonstrated by LUXTURNA in our clinical trials may not be sustained over extended periods of time; (iv) 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; (v) our early preliminary clinical results for our product candidate, *SPK-8011*, for hemophilia A may not be sustained or sufficient to support further development; (vi) we may be unsuccessful in achieving higher factor VIII activity levels through dose escalation in our phase 1/2 clinical trial of *SPK-8011*; (vii) our lead *SPK-FIX* product candidate, *SPK-9001*, may not produce sufficient data in our Phase 1/2 clinical trial to warrant further development; (viii) our early preliminary data in our phase 1/2 clinical trial of *SPK-8011* have yet to be audited and therefore are subject to confirmation in connection with a clinical trial audit; and (ix) 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" 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 | June 30, 2017 |
|---|---|---|
| **Assets** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 58,923,097 | $ 36,811,703 |
| Marketable securities | 237,242,655 | 178,983,007 |
| Other receivables | 16,780,917 | 5,009,699 |
| Prepaid expenses and other current assets | 1,647,008 | 5,519,555 |
| Total current assets | 314,593,677 | 226,323,964 |
| Marketable securities | 21,900,129 | 22,768,329 |
| Property and equipment, net | 19,794,306 | 25,530,072 |
| Acquired-in-process research and development | 15,490,000 | — |
| Goodwill | 1,160,104 | 1,195,754 |
| Other assets | 924,579 | 814,283 |
| Total assets | $ 373,862,795 | $ 276,632,402 |
| **Liabilities and Stockholders' Equity** | | |
| Current liabilities: | | |
| Accounts payable | $ 9,928,737 | $ 15,103,921 |
| Accrued expenses and other | 13,826,920 | 14,432,975 |
| Current portion of long-term debt | 302,013 | 306,954 |
| Current portion of deferred rent | 771,196 | 1,033,370 |
| Current portion of deferred revenue | 5,168,674 | 6,276,859 |
| Total current liabilities | 29,997,540 | 37,154,079 |
| Long-term debt | 1,224,003 | 1,069,281 |
| Long-term deferred rent | 7,498,419 | 10,250,088 |
| Long-term deferred revenue | 3,865,885 | — |
| Deferred tax liability | 1,000,235 | — |
| Total liabilities | 43,586,082 | 48,473,448 |
| 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; 31,237,030 shares issued and 31,219,519 shares outstanding as of June 30, 2017 | 30,874 | 31,237 |
| Additional paid-in capital | 583,973,682 | 607,530,146 |
| Accumulated other comprehensive (loss) income | (794,296) | 651,042 |
| Treasury stock, at cost, 9,206 shares as of December 31, 2016 and 17,511 shares as of June 30, 2017 | (552,636) | (1,023,447) |
| Accumulated deficit | (252,380,911) | (379,030,024) |
| Total stockholders' equity | 330,276,713 | 228,158,954 |
| Total liabilities and stockholders' equity | $ 373,862,795 | $ 276,632,402 |

**Spark Therapeutics, Inc.**
**Consolidated Statements of Operations**
**(Unaudited)**

| | Three months ended June 30, | | Six months ended June 30, | |
|---|---|---|---|---|
| | 2016 | 2017 | 2016 | 2017 |
| Revenues | $ 1,288,629 | $ 1,483,233 | $ 2,577,257 | $ 2,757,700 |
| Operating expenses: | | | | |
| Research and development | 19,621,536 | 32,989,267 | 37,873,436 | 65,337,516 |
| Acquired in-process research and development | — | 3,070,358 | — | 3,457,142 |
| Impairment of acquired in-process research and development | — | 15,696,017 | — | 15,696,017 |
| General and administrative | 10,676,752 | 26,728,606 | 19,550,613 | 48,142,424 |
| Total operating expenses | 30,298,288 | 78,484,248 | 57,424,049 | 132,633,099 |
| Loss from operations | (29,009,659) | (77,001,015) | (54,846,792) | (129,875,399) |
| Interest income, net | 333,544 | 532,509 | 593,966 | 1,117,561 |
| Loss before income taxes | (28,676,115) | (76,468,506) | (54,252,826) | (128,757,838) |
| Income tax benefit | — | 2,108,725 | — | 2,108,725 |
| Net loss | $ (28,676,115) | $ (74,359,781) | $ (54,252,826) | $ (126,649,113) |
| Basic and diluted net loss per common share | $ (1.04) | $ (2.40) | $ (2.00) | $ (4.10) |
| Weighted average basic and diluted common shares outstanding | 27,456,954 | 30,968,450 | 27,132,288 | 30,870,740 |

# # #