Spark Therapeutics, Inc. Form DEF 14A April 21, 2016

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

SCHEDULE 14A

Proxy Statement Pursuant to Section 14(a) of the Securities Exchange Act of 1934

Filed by Registrant x Filed by a Party other than the Registrant: o

Check the appropriate box:

- oPreliminary Proxy Statement
- o Confidential, for Use of the Commission Only (as permitted by Rule 14a-6(e)(2))
- x Definitive Proxy Statement
- oDefinitive Additional Materials
- o Soliciting Material Pursuant to §240.14a-12

Spark Therapeutics, Inc.

(Exact Name of Registrant as Specified in its Charter)

(Name of Person(s) Filing Proxy Statement, if other than the Registrant)

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- (1) Amount Previously Paid:
- (2) Form, Schedule or Registration Statement No.:
- (3) Filing Party:
- (4) Date Filed:

April 21, 2016

Dear Stockholder:

You are cordially invited to attend the 2016 Annual Meeting of Stockholders, or Annual Meeting, of Spark Therapeutics, Inc., or the Company or Spark. The meeting will be held on June 1, 2016 at 8:30 a.m. Eastern Daylight Time, or EDT, at the Company's offices, located at 3737 Market Street, Suite 1300, Philadelphia, PA 19104. Details regarding attendance at the meeting and the business to be conducted are more fully described in the accompanying Notice of Annual Meeting and Proxy Statement.

At the Annual Meeting, the agenda includes the election of three (3) Class I directors for a three (3) year term and the ratification of the appointment of KPMG LLP as the Company's independent registered public accounting firm for the fiscal year ending December 31, 2016.

Under Securities and Exchange Commission rules, the Company is providing access to the proxy materials for the Annual Meeting via the Internet. Accordingly, you can access the proxy materials and vote at www.proxyvote.com. Instructions for accessing the proxy materials and voting are described below and in the Notice of Annual Meeting that you received in the mail. Your vote is very important. Whether or not you plan to attend the meeting, please carefully review the enclosed proxy statement and then cast your vote, regardless of the number of shares you hold. If you are a stockholder of record, you may vote over the Internet, by telephone or, if you request to receive a printed set of the proxy materials, by completing, signing, dating and mailing the accompanying proxy card in the return envelope. Submitting your vote via the Internet or by telephone or proxy card will not affect your right to vote in person if you decide to attend the Annual Meeting. If your shares are held in street name (held for your account by a broker or other nominee), you will receive instructions from your broker or other nominee explaining how to vote your shares and you will have the option to cast your vote by telephone or over the Internet if your voting instruction form from your broker or nominee includes instructions and a toll-free telephone number or Internet website to do so. In any event, to be sure that your vote will be received in time, please cast your vote by your choice of available means at your earliest convenience.

Your continuing interest in the Company is very much appreciated. Sincerely,

/s/ Jeffrey D. Marrazzo Jeffrey D. Marrazzo Chief Executive Officer

NOTICE OF 2016 ANNUAL MEETING OF STOCKHOLDERS

Time 8:30 a.m., EDT

Date June 1, 2016

Place The offices of Spark Therapeutics, Inc., located at 3737 Market Street, Suite 1300, Philadelphia, PA 19104.

Purpose

- 1. To elect A. Lorris Betz, M.D., Ph.D., Anand Mehra, M.D. and Lota Zoth as Class I members of the Board of Directors, to serve until their successors are duly elected and qualified;
- 2. To ratify the selection of KPMG LLP as the Company's independent registered public accounting firm for the fiscal year ending December 31, 2016; and
- 3. To transact any other business that may properly come before the Annual Meeting or any adjournment thereof.

Record Date

The Board of Directors has fixed the close of business on April 7, 2016 as the record date for determining stockholders entitled to notice of and to vote at the meeting.

Meeting Attendance All stockholders as of the record date, or their duly appointed proxies, are invited to attend the meeting. If you attend, you will be asked to present valid picture identification such as a driver's license or passport.

Voting by Proxy

If you are a stockholder of record, please vote via the Internet or, for shares held in street name, please submit the voting instruction form you receive from your broker or nominee as soon as possible so your shares can be voted at the meeting. You may submit your voting instruction form by mail. If you are a stockholder of record, you also may vote by telephone or by submitting a proxy card by mail. If your shares are held in street name, you will receive instructions from your broker or other nominee explaining how to vote your shares, and you also may have the choice of instructing the record holder as to the voting of your shares over the Internet or by telephone. Follow the instructions on the voting instruction form you received from your broker or nominee.

By order of the Board of Directors,

/s/ Joseph W. La Barge Joseph W. La Barge Secretary Philadelphia, Pennsylvania April 21, 2016

Important notice regarding the availability of proxy materials for the Spark Therapeutics, Inc. 2016 Annual Meeting of Stockholders to be held on June 1, 2016: The Notice of 2016 Annual Meeting of Stockholders, proxy statement and our Annual Report on Form 10-K for the fiscal year ended December 31, 2015 are available at www.sparktx.com by following the link for "Investors". To obtain directions to the Company's offices in order to attend the Annual Meeting in person, please visit the "Investors - Events" section of our website at www.sparktx.com or contact Investor Relations at (855) 772-7589.

TABLE OF CONTENTS

	PAGE
Information about the Annual Meeting and Voting	<u>1</u>
Votes Required	<u>3</u>
Proposal 1: Election of Class I Directors	<u>4</u>
Director Biographies	<u>5</u>
How Our Board is Organized	<u>7</u>
Director Independence	<u>8</u>
Board Committees	<u>8</u>
Board Meetings and Attendance	<u>10</u>
Board Processes	<u>11</u>
Policies and Procedures for Related-Person Transactions	<u>12</u>
Stockholder Proposals	<u>13</u>
Executive Officers	<u>13</u>
Executive Compensation	13 13 14 17 23 23 25
Equity Incentive and Other Compensation Plans	<u>17</u>
Risk Considerations in Our Compensation Program	<u>23</u>
Director Compensation	<u>23</u>
Security Ownership of Certain Beneficial Owners, Management and Related Stockholders	<u>25</u>
Certain Relationships and Related Transactions and Director Independence	<u>26</u>
Audit Committee Report	<u>28</u>
Proposal 2: Ratification of Selection of Independent Registered Public Accounting Firm	<u>28</u>
Auditor's Fees	<u>29</u>
Policies on Reporting of Concerns Regarding Accounting and Other Matters and On Communicating with	<u>29</u>
Non-Management Directors	<u>2)</u>
Where You Can Find More Information	<u>30</u>
Form 10-K	<u>30</u>
Householding of Annual Meeting Materials	<u>31</u>
Other Business	<u>31</u>

SPARK THERAPEUTICS, INC.
3737 MARKET STREET
THIRTEENTH FLOOR
PHILADELPHIA, PA 19104
PROXY STATEMENT
FOR THE 2016 ANNUAL MEETING OF STOCKHOLDERS
TO BE HELD ON JUNE 1, 2016
AT 8:30 AM EDT

INFORMATION ABOUT THE ANNUAL MEETING AND VOTING

incorporated by reference in, or considered to be a part of, this document.

When is this proxy statement and the accompanying materials scheduled to be sent to stockholders? We have elected to provide access to our proxy materials for the Annual Meeting to our stockholders via the Internet. Accordingly, on or about April 21, 2016 we will begin mailing a Notice of Internet Availability of Proxy Materials, or Notice of Internet Availability, and all other proxy materials, including the Notice of 2016 Annual Meeting of Stockholders, this proxy statement and accompanying proxy card. For shares held in street name (held for your account by a broker or other nominee), a voting instruction form and the Annual Report on Form 10-K for the fiscal year ending December 31, 2015 will be made available to stockholders on the Internet on the same date. Why did I receive a Notice of Internet Availability of Proxy Materials instead of a full set of proxy materials? Pursuant to rules adopted by the Securities and Exchange Commission, or SEC, we are providing access to our proxy materials over the Internet rather than printing and mailing the proxy materials. We believe electronic delivery will expedite the receipt of materials and will help lower our costs and reduce the environmental impact of our Annual Meeting materials. Therefore, a Notice of Internet Availability will be mailed to holders of record and beneficial owners of our common stock starting on or around April 21, 2016. The Notice of Internet Availability will provide instructions as to how stockholders may access and review the proxy materials, including the Notice of Annual Meeting, proxy statement, proxy card and Annual Report on Form 10-K, on the website referred to in the Notice of Internet Availability or, alternatively, how to request that a copy of the proxy materials, including a proxy card, be

Who is soliciting my vote?

The Board of Directors of Spark, or Board of Directors, is soliciting your vote for the Annual Meeting. When is the record date for the Annual Meeting?

sent to them by mail. The Notice of Internet Availability also will provide voting instructions. In addition,

stockholders of record may request to receive the proxy materials in printed form by mail or electronically by e-mail on an ongoing basis for future stockholder meetings. Please note that, while our proxy materials are available at the website referenced in the Notice of Internet Availability, and our Notice of Annual Meeting, proxy statement and Annual Report on Form 10-K are available on our website, no other information contained on either website is

The Company's Board of Directors has fixed the record date for the Annual Meeting as of the close of business on April 7, 2016.

How many votes can be cast by all stockholders?

A total of 27,379,787 shares of common stock of the Company were outstanding on April 7, 2016 and entitled to be voted at the Annual Meeting. Each share of common stock is entitled to one vote on each matter.

How do I vote?

If you are a stockholder of record and your shares are registered directly in your name, you may vote:
By Internet. Access the website of the Company's tabulator, Broadridge, at: www.proxyvote.com, using the voter control number printed on the furnished proxy card. Your shares will be voted in accordance with your instructions.

You must specify how you want your shares voted or your Internet vote cannot be completed and you will receive an error message. If you vote on the Internet, you also may request electronic delivery of future proxy materials.

By Telephone. Call 1-800-690-6903 toll-free from the U.S., U.S. territories and Canada and follow the instructions on the enclosed proxy card. Your shares will be voted in accordance with your instructions. You must specify how you want your shares voted or your telephone vote cannot be completed.

By Mail. Complete and mail a proxy card in the enclosed postage prepaid envelope to Broadridge. Your proxy will be voted in accordance with your instructions. If you sign and return the enclosed proxy but do not specify how you want your shares voted, they will be voted FOR the nominees named herein to the Company's Board of Directors and FOR the ratification of KPMG LLP as the Company's independent registered public accounting firm for the fiscal year ending December 31, 2016 and will be voted according to the discretion of the proxy holder upon any other business that may properly be brought before the meeting and at all adjournments and postponements thereof. If you are mailed or otherwise receive or obtain a proxy card, and you choose to vote by telephone or by Internet, you do not have to return your proxy card.

In Person at the Meeting. If you attend the meeting, be sure to bring a form of personal picture identification with you, and you may deliver your completed proxy card in person, or you may vote by completing a ballot, which will be available at the meeting.

Telephone and Internet voting for stockholders of record will be available until 11:59 p.m. EDT the day before the Annual Meeting, and mailed proxy cards must be received by the day before the Annual Meeting to be counted. If the Annual Meeting is adjourned or postponed, these deadlines may be extended.

If your shares of common stock are held in street name (held for your account by a broker or other nominee):

By Internet or By Telephone. You will receive instructions or a voting instruction form from your broker or other nominee if you are permitted to vote by Internet or telephone.

By Mail. You will receive instructions from your broker or other nominee explaining how to vote your shares. In Person at the Meeting. If you attend the meeting, in addition to picture identification you should both bring an account statement or a letter from the record holder indicating that you owned the shares as of the record date, and contact the broker or other nominee who holds your shares to obtain a broker's proxy card and bring it with you to the meeting.

What are the Board's recommendations on how to vote my shares?

The Board of Directors recommends a vote:

Proposal 1: FOR election of the three Class I directors (page 4)

Proposal 2: FOR ratification of the selection of KPMG LLP as the Company's independent registered public accounting firm (page 29)

Who pays the cost for soliciting proxies?

The Company will pay the cost for the solicitation of proxies by the Board of Directors. The solicitation of proxies will be made primarily by mail and through internet access to materials. Proxies also may be solicited personally, by telephone, fax or e-mail by employees of the Company without any remuneration to such individuals other than their regular compensation. The Company also will reimburse brokers, banks, custodians, other nominees and fiduciaries for forwarding these materials to their principals to obtain the authorization for the execution of proxies.

How are proxies voted?

All shares represented by valid proxies received prior to the applicable deadlines will be voted and where a shareholder specifies by means of the proxy a choice with respect to any matter to be acted upon, the shares will be voted in accordance with the shareholder's instructions. However, if no choice is specified on a proxy as to one or more of the proposals, the proxy will be voted in accordance with the Board of Directors' recommendations on such proposals as set forth in this proxy statement.

Can I change my vote?

After you have submitted your proxy, you may still change your vote and revoke your proxy prior to the Annual Meeting by taking any one of the following actions:

submitting a new proxy by following the "Vote by Internet" or "Vote by Phone" instructions on the enclosed proxy card up until 11:59 p.m., EDT, the day before the Annual Meeting;

signing another proxy card and either arranging for delivery of that proxy card by mail prior to the start of the Annual Meeting or by delivering that signed proxy card in person at the Annual Meeting;

giving our Secretary a written notice before or at the Annual Meeting that you want to revoke your proxy; or voting in person at the Annual Meeting.

Your attendance at the Annual Meeting alone will not revoke your proxy. If the shares you own are held in "street name" by a bank, broker or other nominee record holder, which we collectively refer to in this proxy statement as "brokerage firms", your brokerage firm, as the record holder of your shares, is required to vote your shares according to your instructions.

Could other matters be decided at the Annual Meeting?

The Company does not know of any other matters that may be presented for action at the Annual Meeting. Should any other business come before the meeting, the persons named on the enclosed proxy will have discretionary authority to vote the shares represented by such proxies in accordance with their best judgment. If you hold shares through a broker, bank or other nominee as described above, they will not be able to vote your shares on any other business that comes before the Annual Meeting unless they receive instructions from you with respect to such matter.

What happens if the meeting is postponed or adjourned?

Your proxy may be voted at the postponed or adjourned meeting. You will still be able to change your proxy as outlined above under "Can I change my vote?".

How can I find out the results of the voting at the Annual Meeting?

Preliminary voting results will be announced at the Annual Meeting. Final voting results will be published in a Current Report on Form 8-K, or Form 8-K, that we expect to file with the SEC within four business days after the Annual Meeting.

What does it mean if I receive more than one proxy card or voting instruction form?

It means that you have multiple accounts at the transfer agent or with brokers. Please complete and return all proxy cards or voting instruction forms to ensure that all of your shares are voted.

Implications of being an "emerging growth company".

We are an "emerging growth company" as that term is used in the Jumpstart Our Business Startups (JOBS) Act of 2012 and, as such, have elected to comply with certain reduced public company reporting requirements. These reduced reporting requirements include reduced disclosure about the Company's executive compensation arrangements and no non-binding advisory votes on executive compensation. We will remain an emerging growth company until the earliest of (1) December 31, 2020, (2) the last day of the fiscal year in which we have total annual gross revenue of at least \$1.0 billion, (3) the last day of the fiscal year in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds \$700 million as of the prior June 30th, and (4) the date on which we have issued more than \$1.0 billion in non-convertible debt during the prior three-year period.

Who should I call if I have any additional questions?

If you hold your shares directly, please call Joseph W. La Barge, Secretary of the Company, at (855) 772-7589. If your shares are held in street name, please call the telephone number provided on your voting instruction form or contact your broker or nominee holder directly.

VOTES REQUIRED

What vote is required to approve each item?

The three nominees for election as Class I directors who receive the highest number of the votes cast (called a plurality) for election of directors shall be elected directors (Proposal 1). A majority of votes cast is necessary for ratification of the selection of KPMG LLP as the Company's independent registered public accounting firm for the fiscal year ending December 31, 2016 (Proposal 2).

If there are insufficient votes to approve Proposal 2, your proxy may be voted by the persons named in the proxy to adjourn the Annual Meeting in order to solicit additional proxies in favor of the approval of such proposal. If the Annual Meeting is adjourned or postponed for any purpose, at any subsequent reconvening of the meeting, your proxy will be voted in the same manner as it would have been voted at the original convening of the Annual Meeting unless you withdraw or revoke your proxy. Your proxy may be voted in this manner even though it may have been voted on the same or any other matter at a previous session of the Annual Meeting.

How is a quorum reached?

The presence, in person or by proxy, of holders of at least a majority of the total number of outstanding shares entitled to vote is necessary to constitute a quorum for the transaction of business at the Annual Meeting. Shares held of record by stockholders or brokers, bankers or other nominees who do not return a signed and dated proxy or attend the Annual Meeting in person will not be considered present or represented at the Annual Meeting and will not be counted in determining the presence of a quorum. Abstentions and "broker non-votes", if any, will be counted for purposes of determining whether a quorum is present for the transaction of business at the meeting. Broker non-votes occur when your broker or other nominee submits proxy for your shares (because the broker or nominee has received instructions from you on one or more proposals, but not all, or has not received instructions from you but is entitled to vote on a particular "discretionary" matter) but does not indicate a vote for a particular proposal because the broker or other nominee does not have the authority to vote on that proposal and has not received voting instructions from you or has discretionary authority but chooses not to exercise it. If a quorum is not present, the meeting may be adjourned until a quorum is obtained.

How is the vote counted?

Votes cast by proxy or in person at the Annual Meeting will be counted by the persons appointed by Spark to act as tabulators for the meeting. The tabulators will count all votes "for," "against," or "withhold," as well as abstentions and broker non-votes, as applicable, for each matter to be voted on at the Annual Meeting. Shares represented by proxies that withhold authority to vote for a nominee for election as a director will not be counted as votes "for" a director. Shares properly voted to "abstain" on a particular matter and broker non-votes are treated as having not voted on the particular matter and will therefore not affect the outcome of Proposal 2.

Under the rules that govern brokers holding shares for their customers, brokers who do not receive voting instructions from their customers have the discretion to vote uninstructed shares on routine matters, but do not have discretion to vote such uninstructed shares on non-routine matters. Only Proposal 2, the ratification of the selection of KPMG LLP, is considered a routine matter where brokers are permitted to vote shares held by them without instruction. If your shares are held through a broker, those shares will not be voted in the election of directors unless you affirmatively provide the broker instructions on how to vote.

PROPOSAL 1: ELECTION OF CLASS I DIRECTORS

In accordance with Delaware law and the Company's Restated Certificate of Incorporation and By-laws Amended and Restated, the Board of Directors is divided into three classes of approximately equal size, with members of each class serving staggered three year terms. A. Lorris Betz, M.D., Ph.D., Anand Mehra, M.D. and Lota Zoth, CPA are the directors whose terms expire at this Annual Meeting and each of Drs. Betz and Mehra and Ms. Zoth has been nominated for and has agreed to stand for re-election to the Board of Directors to serve as a Class I director of the Company until the annual meeting of stockholders to be held in 2019 or and until his or her successor is duly elected. Pursuant to the By-laws, the Board of Directors has fixed the number of directors at nine (9) as of the date of the Annual Meeting.

It is intended that, unless you give contrary instructions, shares represented by proxies solicited by the Board of Directors will be voted for the election of the three nominees listed below as director nominees. The vote of a plurality of the votes cast at the

meeting will be required for the election of the Class I director nominees. Broker non-votes and votes withheld will not be treated as votes cast for this purpose and, therefore, will not affect the outcome of the election. The Company has no reason to believe that any nominee will be unavailable for election at the Annual Meeting. In the event that one or more nominees is unexpectedly not available to serve, proxies may be voted for another person nominated as a substitute by the Board of Directors, or the Board of Directors may reduce the number of directors to be elected at the Annual Meeting. Information relating to each nominee for election as director and for each continuing director, including his or her period of service as a director of the Company, principal occupation and other biographical material is shown under the heading "Director Biographies".

THE BOARD OF DIRECTORS UNANIMOUSLY RECOMMENDS THAT YOU VOTE **FOR**

EACH OF THESE NOMINEES FOR CLASS I DIRECTOR (PROPOSAL 1 ON YOUR PROXY CARD)

A. Lorris Betz, M.D., Ph.D. Anand Mehra, M.D. Lota Zoth, CPA

DIRECTOR BIOGRAPHIES

The following table sets forth information concerning each member of our Board of Directors as of April 7, 2016. The biographical description of each director includes the specific experience, qualifications, attributes and skills that the Board of Directors would expect to consider if it were making a conclusion currently as to whether such person should serve as a director. In addition to the specific qualifications enumerated in their biographies, we believe that each of our directors possesses the attributes an characteristics described below under the heading "Board Processes -Minimum qualifications".

NAME	Age	Position
Jeffrey D. Marrazzo	37	Co-founder, Chief Executive Officer and Director
Katherine A. High, M.D. (4)	64	Co-founder, President, Chief Scientific Officer and Director
Steven Altschuler, M.D. (3)	62	Chairman of the Board of Directors
A. Lorris Betz, M.D., Ph.D.	69	Director
Lars Ekman, M.D., Ph.D. (1) (3) (4)	66	Director
Anand Mehra, M.D. (1) (2) (4)	40	Director
Vincent J. Milano (1) (2)	52	Director
Elliott Sigal, M.D., Ph.D. (2) (3) (4)	64	Director
Lota Zoth, CPA (1)	56	Director
(1) Member of the audit committee.		

- (1) Member of the audit committee.
- (2) Member of the compensation committee.
- (3) Member of the nominating and corporate governance committee.
- (4) Member of the science and technology committee.

Jeffrey D. Marrazzo

Jeffrey D. Marrazzo is a co-founder and has served as Chief Executive Officer of Spark and as a member of our Board of Directors since our founding in 2013. Prior to founding Spark, Mr. Marrazzo launched and was Chief Business Officer of the U.S. division of Molecular Health, Inc. from 2011 to 2013. Mr. Marrazzo was part of the founding management of Generation Health from 2009 to 2011, up to and through the acquisition of a majority of the company's shares by CVS Caremark. From 2008 to 2009, Mr. Marrazzo served as an employee and independent consultant to the business development and finance teams at Tengion Inc. and, from 2011 to 2013, Mr. Marrazzo served as an independent consultant to the Children's Hospital of Philadelphia, or CHOP. Previously, Mr. Marrazzo served as healthcare advisor to former Pennsylvania Governor Edward G. Rendell and as an IBM management consultant to

global pharmaceutical companies. Mr. Marrazzo holds a B.S.E. and B.A. in systems science and engineering and economics from the University of Pennsylvania and a dual M.B.A./M.P.A. from The Wharton School and Harvard University. We believe that Mr. Marrazzo is qualified to serve on our Board of Directors because

of his extensive leadership experience in the life sciences industry and his extensive knowledge of our Company based on his role as co-founder and Chief Executive Officer.

Katherine A. High, M.D.

Dr. Katherine A. High is a co-founder and has served as our President and Chief Scientific Officer and a member of our Board of Directors since September 2014. Prior to serving as our President, Dr. High provided advice to Spark and subsequently served as an independent consultant to Spark from December 2013 to September 2014. Dr. High was previously a Professor at the Perelman School of Medicine at the University of Pennsylvania and an Investigator of the Howard Hughes Medical Institute. She served as the Director of the Center for Cellular and Molecular Therapeutics at CHOP from 2004 to 2014, where her team's research led to the discovery of new gene and cell therapies for genetic diseases. Dr. High began her independent research career at Yale University and the University of North Carolina studying the molecular basis of blood coagulation and the development of novel therapeutics for the treatment of bleeding disorders. Dr. High's studies at CHOP established the first proof of principle of gene therapy for hemophilia in preclinical models and led to a series of studies that characterized the human immune response to AAV vectors in a variety of target tissues. Dr. High served a five-year term from 2000 to 2005 on the FDA Advisory Committee on Cell, Tissue and Gene Therapies and is a past-president of the American Society of Gene & Cell Therapy. Dr. High holds an A.B. in chemistry from Harvard, an M.D. from the University of North Carolina School of Medicine, a business certification from the University of North Carolina Business School Management Institute for Hospital Administrators and an honorary M.A. from the University of Pennsylvania. We believe that Dr. High is qualified to serve on our Board of Directors because of her extensive executive and scientific leadership in the life sciences industry and her extensive knowledge of our Company based on her role as co-founder, President and Chief Scientific Officer.

Non-Employee Directors

Steven M. Altschuler, M.D.

Dr. Steven M. Altschuler has served on our Board of Directors and has been the Chairman of our board since October 2013. Dr. Altschuler became Senior Vice President of Health Affairs at the University of Miami and Chief Executive Officer of UHealth-University of Miami Health System in January 2016. Previously, he was Chief Executive Officer of CHOP and The Children's Hospital of Philadelphia Foundation, or the CHOP Foundation, from 2000 to 2015. Prior to serving as the Chief Executive Officer of CHOP, Dr. Altschuler served in many leadership roles at CHOP including: Division Chief of Gastroenterology, Physician-in-Chief, inaugural holder of the Leonard and Madlyn Abramson Endowed Chair in Pediatrics and Professor and Chair of the Department of Pediatrics at the Perelman School of Medicine at the University of Pennsylvania, where he was a faculty member from 1985 to 2000. Dr. Altschuler has served on the board of directors for Mead Johnson Nutrition Company since 2009 and Weight Watchers International since 2012. Dr. Altschuler holds a B.A. in mathematics and an M.D., both from Case Western Reserve University. He completed his pediatric internship and residency at Children's Hospital Medical Center-Boston and fellowship training in gastroenterology and nutrition at CHOP and the University of Pennsylvania School of Medicine. We believe that Dr. Altschuler is qualified to serve on our Board of Directors because of his extensive experience in the medical industry, his service on the boards of directors of other life sciences companies and his extensive leadership experience.

A. Lorris Betz, M.D., Ph.D.

Dr. A. Lorris Betz has served on our Board of Directors since January 2015. Dr. Betz served as Senior Vice President for Health Sciences and Dean of the School of Medicine at The University of Utah, where he also was a faculty member, and as Chief Executive Officer of the University of Utah Health Care, from 1999 to 2011. Dr. Betz also served twice as the Interim President of the University of Utah from January 2004 to August 2004 and from May 2011 to March 2012. Dr. Betz previously held various faculty positions at the University of Michigan from 1979 to 1998. Dr. Betz has served on the board of directors of the Association of Medical Colleges since 2012, including as Chairman from 2013 to 2014, and currently serves as a member of the Board of Trustees and Executive Committee of CHOP and the CHOP Foundation. Dr. Betz holds a B.S., M.D. and Ph. D. in biochemistry and physiology from the University of Wisconsin. He completed his pediatric residency and a research fellowship in pediatric neurology at the University of California, San Francisco. We believe that Dr. Betz is qualified to serve on our Board of Directors

because of his extensive experience in medicine and the medical industry and his extensive leadership experience. Lars Ekman, M.D., Ph.D.

Dr. Lars Ekman has served on our Board of Directors since May 2014. He is currently Executive Partner at Sofinnova Ventures which he joined in 2008. Prior to joining Sofinnova Ventures, Dr. Ekman was President of Research and Development at Elan Corporation (now Perrigo) from January 2001 to December 2007. Prior to Elan, he was Executive Vice President, Research and Development, at Schwarz Pharma AG (now UCB) and, prior to then, held a variety of senior scientific and clinical roles at Pharmacia (now Pfizer). Dr. Ekman is Chairman of Amarin Corporation, Chairman of Prothena Biosciences and Chairman of

Sophiris Bioscience. He also serves on the board of directors of Ultragenyx. He co-founded and served as Chief Executive Officer of Cebix, Inc., and served on its board from 2009 to 2015 as well as on the board of directors of InterMune, Inc. from 2006-2015, Elan Plc from 2005-2013 and Ocera Therapeutics, Inc. from 2009-2015. Dr. Ekman is a board-certified surgeon and holds an M.D. and a Ph.D. in experimental biology from the University of Gothenburg, Sweden. We believe that Dr. Ekman is qualified to serve on our Board of Directors because of his extensive experience in the life sciences industry, both as an executive and as a venture capital investor, in addition to his extensive leadership experience.

Anand Mehra, M.D.

Dr. Anand Mehra has served on our Board of Directors since May 2014. Dr. Mehra is currently a General Partner of Sofinnova Ventures, which he joined in 2007. Prior to joining Sofinnova, Dr. Mehra worked in J.P. Morgan's private equity and venture capital group, and before that, Dr. Mehra was a consultant in McKinsey & Company's pharmaceutical practice. Dr. Mehra has served on the board of directors of Aerie Pharmaceuticals since August 2010, Aclaris Therapeutics, Inc. since 2014 and Marinus Pharmaceuticals since October 2007 as well as several private companies. Dr. Mehra holds a B.A. from the University of Virginia and an M.D. from Columbia University's College of Physicians and Surgeons. We believe that Dr. Mehra is qualified to serve on our Board of Directors because of his extensive experience in the life sciences industry, his service on the boards of directors of other life sciences companies and his extensive leadership experience.

Vincent J. Milano

Vincent J. Milano has served on our Board of Directors since June 2014. Mr. Milano is currently the Chief Executive Officer of Idera Pharmaceuticals Inc. Prior to joining Idera, Mr. Milano served as President, Chief Executive Officer and Chairman of the board of directors of ViroPharma Incorporated, or ViroPharma, from 2008 to 2014, which was acquired by Shire Pharmaceuticals in January 2014. Mr. Milano joined ViroPharma in 1996 and served as Vice President, Chief Financial Officer and Treasurer from 1997 to 2006. Prior to joining ViroPharma, Mr. Milano served as a Senior Manager at KPMG LLP, independent certified public accountants. Mr. Milano has served on the board of directors of Vanda Pharmaceuticals since 2010 and the board of directors of VenatoRx, a privately held biotechnology company, since 2013. He also serves on the board of directors of Pennsylvania Bio. Mr. Milano holds a B.S. in accounting from Rider College. We believe that Mr. Milano is qualified to serve on our Board of Directors because of his extensive experience in the life sciences industry, his financial expertise and his extensive leadership experience. Elliott Sigal, M.D., Ph.D.

Dr. Elliott Sigal has served on our Board of Directors since January 2014. Dr. Sigal served as Chief Scientific Officer of Bristol-Myers Squibb (BMS) from 2004 until his retirement in 2013 and was a member of the board of directors from 2011 to 2013. Dr. Sigal joined BMS in 1997 and held positions of increasing responsibility in drug discovery and development and was a member of the executive committee from 2001-2013. Prior to BMS, he was Vice President of R&D and Chief Executive Officer for the genomics firm Mercator Genetics Inc. Dr. Sigal currently serves as a member of the board of directors for Adaptimmune Therapeutics plc and the Mead Johnson Nutrition Company. In addition, Dr. Sigal is an advisor to the life sciences venture firm, New Enterprise Associates, and consults for select biopharmaceutical companies. Dr. Sigal holds B.S., M.S. and Ph.D. degrees in industrial engineering from Purdue University and an M.D. from the University of Chicago. He completed his training in internal medicine and pulmonary medicine at the University of California, San Francisco. He received his research training at the Cardiovascular Research Institute at UCSF, where he served on the faculty of the UCSF Department of Medicine. We believe that Dr. Sigal is qualified to serve on our Board of Directors because of his extensive experience in the life sciences industry and his extensive leadership experience.

Lota Zoth, CPA

Lota Zoth has served on our Board of Directors since January 2016. Ms. Zoth has served in senior financial roles in a variety of commercial-stage companies over a 30-year career, including serving as Chief Financial Officer for MedImmune before its acquisition by AstraZeneca in 2007. Prior to joining MedImmune in 2002, Ms. Zoth served as Senior Vice President, Corporate Controller and Principal Accounting Officer at PSINet Inc., Vice President, Corporate Controller and Chief Accounting Officer at Sodexho Marriott Services, Inc., Marriott International and PepsiCo, Inc. Ms. Zoth also served as an auditor at Ernst & Young, LLP and is a Certified Public Accountant. Ms.

Zoth received a B.B.A. in accounting from Texas Tech University. She currently serves on the board of Orexigen Therapeutics, Inc., Aeras, Circassia Pharmaceuticals, PLC and NewLink Genetics Corporation. We believe that Ms. Zoth is qualified to serve on our Board of Directors because of her extensive experience in the life sciences industry, her financial expertise and extensive leadership experience.

HOW OUR BOARD IS ORGANIZED

Composition of the Board of Directors

Our Board of Directors currently is authorized to have nine members and is divided into three classes, Class I, Class II and Class III, with members of each class serving staggered three-year terms. The members of the classes are as follows:

the Class I directors are Drs. Betz and Mehra and Ms. Zoth and their term expires at the 2016 Annual Meeting; the Class II directors are Drs. Altschuler, Ekman and High and their term expires at the annual meeting of stockholders to be held in 2017; and

the Class III directors are Messrs. Marrazzo and Milano and Dr. Sigal and their term expires at the annual meeting of stockholders to be held in 2018.

Upon the expiration of the term of a class of directors, directors in that class are eligible to be elected for a new three-year term at the annual meeting of stockholders in the year in which their term expires. In accordance with the terms of our Restated Certificate of Incorporation and Amended and Restated Bylaws, our directors may be removed only for cause by the affirmative vote of the holders of at least 75% of the votes that all our stockholders would be entitled to cast in an annual election of directors.

DIRECTOR INDEPENDENCE

Rule 5605 of the NASDAQ Listing Rules requires a majority of a listed company's Board of Directors to be comprised of independent directors within one year of listing. In addition, the NASDAQ Listing Rules require that, subject to specified exceptions, each member of a listed company's audit, compensation and nominating and corporate governance committees be independent and that audit committee members also satisfy independence criteria set forth in Rule 10A-3 under the Securities Exchange Act of 1934, as amended, or the Exchange Act.

Under Rule 5605(a)(2) of the NASDAQ Listing Rules, a director will only qualify as an "independent director" if, in the opinion of our Board of Directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director. In order to be considered independent for purposes of Rule 10A-3, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the Board of Directors or any other board committee: (1) accept, directly or indirectly, any consulting, advisory or other compensatory fee from the listed company or any of its subsidiaries; or (2) be an affiliated person of the listed company or any of its subsidiaries. In addition, in affirmatively determining the independence of any director who will serve on a company's compensation committee, Rule 10C-1 under the Exchange Act requires that a company's Board of Directors consider all factors specifically relevant to determining whether a director has a relationship to such company which is material to that director's ability to be independent from management in connection with the duties of a compensation committee member, including, but not limited to: (1) the source of compensation of the director, including any consulting, advisory or other compensatory fee paid by such company to the director; and (2) whether the director is affiliated with the company or any of its subsidiaries or affiliates.

In March 2016, our Board of Directors undertook a review of the composition of our Board of Directors and its committees and the independence of each director. Based upon information requested from, and provided by, each director concerning his or her background, employment and affiliations, including family relationships, our Board of Directors has determined that each of our directors, with the exception of Mr. Marrazzo and Dr. High, is an "independent director" as defined under Rule 5605(a)(2) of the NASDAQ Listing Rules. Our Board of Directors also determined that Ms. Zoth, Mr. Milano and Drs. Ekman and Mehra who comprise our audit committee, Mr. Milano and Drs. Mehra and Sigal, who comprise our compensation committee, and Drs. Ekman, Altschuler and Sigal, who comprise our nominating and corporate governance committee, satisfy the independence standards for such committees established by the SEC and the NASDAQ Listing Rules, as applicable. In making such determinations, our Board of Directors considered the relationships that each such non-employee director has with our Company and all other facts and circumstances our Board of Directors deemed relevant in determining independence, including the beneficial ownership of our capital stock by each non-employee director.

There are no family relationships among any of our directors or executive officers.

BOARD COMMITTEES

Our Board has established four standing committees: audit, compensation, nominating and corporate governance and science and technology, each of which operates under a charter that has been approved by our board.

Audit committee

The members of our audit committee are Ms. Zoth, Mr. Milano and Drs. Ekman and Mehra. Ms. Zoth is the chair of the audit committee as of January 6, 2016, prior to which Mr. Milano was the chair of the audit committee. During the fiscal year ended December 31, 2015, the audit committee met six times. The report of the audit committee is included in this Proxy Statement under the heading "Audit Committee Report". The audit committee's responsibilities include:

appointing, approving the compensation of and assessing the independence of our independent registered public accounting firm;

overseeing the work of our independent registered public accounting firm, including through the receipt and consideration of reports from such firm;

reviewing and discussing with management and the independent registered public accounting firm our annual and quarterly financial statements and related disclosures;

monitoring our internal control over financial reporting, disclosure controls and procedures and code of business conduct and ethics, including the Company compliance hotline;

assessing our risk management policies;

establishing policies regarding hiring employees from the independent registered public accounting firm;

procedures for the receipt and retention of accounting related complaints and concerns;

meeting independently with our independent registered public accounting firm, management and internal auditing staff, if any;

reviewing and approving or ratifying any related-person transactions; and

preparing the audit committee report required by SEC rules.

All audit and non-audit services to be provided to us by our independent registered public accounting firm must be approved in advance by our audit committee.

Our Board of Directors has determined that Ms. Zoth is an "audit committee financial expert" as defined by applicable SEC rules. We believe that the composition of our audit committee meets the requirements for independence under the current NASDAQ Listing Rules and SEC rules and regulations.

Compensation committee

The members of our compensation committee are Mr. Milano and Drs. Mehra and Sigal. Mr. Milano is the chair of the compensation committee. During the fiscal year ended December 31, 2015, the compensation committee met five times. Our compensation committee's responsibilities include:

annually reviewing and approving corporate goals and objectives relevant to compensation for the CEO and our other executive officers;

determining our CEO's compensation as well as the compensation of our other executive officers;

overseeing an evaluation of our senior executives;

overseeing and administering our cash and equity incentive plans;

reviewing and making recommendations to our board with respect to director compensation;

reviewing and discussing annually with management our "Compensation Discussion and Analysis" if and when we are required to have such disclosure under SEC rules; and

preparing the annual compensation committee report required by SEC rules.

Our executive compensation program is administered by the compensation committee of our Board of Directors, subject to the oversight and approval of our full Board of Directors. Our compensation committee reviews our executive compensation practices on an annual basis and based on this review approves or, as appropriate, makes recommendations to our Board of Directors for approval of our executive compensation program.

In designing our executive compensation program, our compensation committee considers publicly available compensation data for national and regional companies in the biotechnology/pharmaceutical industry to help guide its executive compensation decisions at the time of hiring and for subsequent adjustments in compensation. In 2015, our compensation committee engaged Towers Watson as its independent compensation consultant, to provide comparative data on executive compensation practices in our industry and to advise on our executive compensation program generally. The committee also engaged Towers Watson for recommendations and review of non-employee director compensation in 2015. Although our compensation committee considers the advice and recommendations of Towers Watson as to our executive compensation program, our compensation committee ultimately makes their own decisions about these matters. In the future, we expect that our compensation committee will continue to engage independent compensation consultants to provide additional guidance on our executive compensation programs and to conduct further competitive benchmarking against a peer group of publicly traded companies.

In July 2015, the compensation committee reviewed information regarding the independence and potential conflicts of interest of Towers Watson, taking into account, among other things, the factors set forth in the NASDAQ listing standards. Based on such review, the committee concluded that the engagement of Towers Watson did not raise any conflict of interest.

Our director compensation program is administered by our Board of Directors with the assistance of the compensation committee. The compensation committee conducts an annual review of director compensation and makes recommendations to the Board of Directors with respect thereto.

Nominating and corporate governance committee

The members of our nominating and corporate governance committee are Drs. Ekman, Altschuler and Sigal. Dr. Ekman is the chair of the nominating and corporate governance committee. During fiscal year ended December 31, 2015, the nominating and corporate governance committee met four times. Our nominating and corporate governance committee's responsibilities include:

*dentifying individuals qualified to become board members;

recommending to our board the persons to be nominated for election as directors and to each of the Board's committees;

reviewing and making recommendations to the board with respect to management succession planning;

- developing and recommending to the board corporate governance principles; and
- overseeing periodic evaluations of the
- board.

Science and technology committee

The members of our science and technology committee are Drs. High, Ekman, Mehra and Sigal. Dr. High is the chair of the science and technology committee. During fiscal year ended December 31, 2015, the science and technology committee met one time. Our science and technology committee's responsibilities include:

reviewing, evaluating and advising the Board of Directors and management regarding the long-term strategic goals and objectives and the quality and direction of our research and development programs;

monitoring and evaluating trends in research and development, and recommending to the Board of Directors and management emerging technologies for building our technological strength;

recommending approaches to acquiring and maintaining technology positions; advising the Board of Directors and management on the scientific aspects of business development transactions; and

regularly reviewing our research and development pipeline.

Compensation committee interlocks and insider participation

Mr. Milano and Drs. Mehra and Sigal served as members of the compensation committee during the year ended December 31, 2015. None of our executive officers serves as a director or a member of a compensation committee (or other committee serving an equivalent function) of any other entity that has one or more of its executive officers

serving as a director or member of our compensation committee. None of the members of our current compensation committee is, or has ever been, an officer or employee of our Company.

BOARD MEETINGS AND ATTENDANCE

Our Board of Directors held nine meetings during the fiscal year ending December 31, 2015. Each of the directors attended at least 75% of the meetings of the Board of Directors and the committees of the Board of Directors on which he or she served during the fiscal year ended December 31, 2015 (in each case, which were held during the period for which he or she was a director and/or a member of the applicable committee). Spark encourages its directors to attend the Annual Meeting of Stockholders. In 2015, we did not hold an annual meeting of stockholders.

As required under applicable NASDAQ listing standards, the Company's independent directors met at least twice in regularly scheduled executive sessions at which only independent directors are present prior to the first anniversary of the Company's initial public offering and in subsequent fiscal years.

BOARD PROCESSES

Identifying and evaluating director nominees

Our Board of Directors is responsible for selecting its own members. The Board delegates the selection and nomination process to the nominating and corporate governance committee, with the expectation that other members of the Board, and of management, will be requested to take part in the process as appropriate. Generally, our nominating and corporate governance committee identifies candidates for director nominees in

Generally, our nominating and corporate governance committee identifies candidates for director nominees in consultation with management, through the use of search firms or other advisors, through the recommendations submitted by stockholders or through such other methods as the nominating and corporate governance committee deems to be helpful to identify candidates. Once candidates have been identified, our nominating and corporate governance committee confirms that the candidates meet all of the minimum qualifications for director nominees established by the nominating and corporate governance committee. The nominating and corporate governance committee may gather information about the candidates through interviews, detailed questionnaires, background checks or any other means that the nominating and corporate governance committee deems to be appropriate in the evaluation process. The nominating and corporate governance committee then meets as a group to discuss and evaluate the qualities and skills of each candidate, both on an individual basis and taking into account the overall composition and needs of our Board of Directors. Based on the results of the evaluation process, the nominating and corporate governance committee recommends candidates for the Board's approval as director nominees for election to the Board.

Minimum qualifications

Our nominating and corporate governance committee will consider, among other things, the following qualifications, skills and attributes when recommending candidates for the Board's selection as nominees for the Board and as candidates for appointment to the Board's committees. The nominee shall have the highest personal and professional integrity, shall have demonstrated exceptional ability and judgment, and shall be most effective, in conjunction with the other nominees to the Board, in collectively serving the long-term interests of the stockholders.

In evaluating proposed director candidates, our nominating and corporate governance committee may consider, in addition to the minimum qualifications and other criteria for board membership approved by the Board from time to time, all facts and circumstances that it deems appropriate or advisable, including, among other things, diversity, and is not limited to race, gender or national origin, the skills of the proposed director candidate, his or her depth and breadth of professional experience or other background characteristics, his or her independence and the needs of the Board. We have no formal policy regarding board diversity, however we give consideration to diversity when evaluating potential new directors. Our nominating and corporate governance committee's priority in selecting board members is identification of persons who will further the interests of our Company through his or her established record of professional accomplishment, the ability to contribute positively to the collaborative culture among board members, and professional and personal experiences and expertise relevant to our growth strategy. The nominating and corporate governance committee will consider candidates recommended by stockholders. The policy adopted by the nominating and corporate governance committee provides that candidates recommended by stockholders are given appropriate consideration in the same manner as other candidates.

Board leadership and the role in risk oversight

Our Board of Directors currently is chaired by Dr. Altschuler. As a general policy, our Board of Directors believes that separation of the positions of chief executive officer and chairman reinforces the independence of the Board of Directors from management, creates an environment that encourages objective oversight of management's performance and enhances the effectiveness of the Board of Directors as a whole. As such, Mr. Marrazzo serves as our Chief Executive Officer while Dr. Altschuler serves as our Chairman of the Board of Directors but is not an officer.

Our Board of Directors oversees the management of risks inherent in the operation of our business and the implementation of our business strategies. Our Board of Directors performs this oversight role by using several different levels of review. In connection with its reviews of the operations and corporate functions of the Company, our Board of Directors addresses the primary risks associated with those operations and corporate functions. In addition, our Board of Directors reviews the risks associated with the Company's business strategies periodically throughout the year as part of its consideration of undertaking any such business strategies.

Each committee of our Board of Directors also oversees the management of the Company's risk that falls within the committee's areas of responsibility. In performing this function, each committee has full access to management, as well as the ability to engage advisors. Our general counsel, principal accounting officer and head of corporate compliance all periodically provide reports to the audit committee and are responsible for identifying, evaluating and implementing risk management controls and methodologies to address any identified risks. In connection with its risk management role, our audit committee meets privately with representatives from our independent registered public accounting firm. The audit committee oversees the operation of our risk management program, including the identification of the primary risks associated with our business and periodic updates to such risks, and reports to our Board of Directors regarding these activities.

Communication with stockholders

Our Board of Directors will give appropriate attention to written communications that are submitted by stockholders, and will respond if and as appropriate. Information regarding how to contact the Board of Directors is available on the Investor Relations section of our website at www.sparktx.com.

POLICIES AND PROCEDURES FOR RELATED-PERSON TRANSACTIONS

On December 29, 2014 our Board of Directors adopted written policies and procedures for the review of any transaction, arrangement or relationship in which we are a participant, the amount involved exceeds \$120,000 and one of our executive officers, director nominees or 5% stockholders (or their immediate family members), each of whom we refer to as a "related person," has a direct or indirect material interest.

If a related person proposes to enter into such a transaction, arrangement or relationship, which we refer to as a "related-person transaction," the related person must report the proposed related-person transaction to our general counsel. The policy calls for the proposed related-person transaction to be reviewed and, if deemed appropriate, approved by the audit committee of our Board of Directors. Whenever practicable, the reporting, review and approval will occur prior to entry into the transaction. If advance review and approval is not practicable, the committee will review and, in its discretion, may ratify the related-person transaction. The policy also permits the chair of the audit committee to review and, if deemed appropriate, approve proposed related-person transactions that arise between committee meetings, subject to ratification by the committee at its next meeting. Any related-person transactions that are ongoing in nature will be reviewed annually.

A related-person transaction reviewed under the policy will be considered approved or ratified if it is authorized by the committee after full disclosure of the related person's interest in the transaction. As appropriate for the circumstances, the committee will review and consider:

the related person's interest in the related-person transaction;

the approximate dollar value of the amount involved in the related-person transaction;

the approximate dollar value of the amount of the related person's interest in the transaction without regard to the amount of any profit or loss;

whether the transaction was undertaken in the ordinary course of our business;

whether the terms of the transaction are no less favorable to us than terms that could have been reached with an unrelated third party;

the purpose of, and the potential benefits to us of, the related-person transaction; and

any other information regarding the related-person transaction or the related person in the context of the proposed transaction that would be material to investors in light of the circumstances of the particular transaction.

The audit committee may approve or ratify the transaction only if the audit committee determines that, under all of the circumstances, the transaction is not inconsistent with our best interests. The audit committee may impose any

conditions on the related-person transaction that it deems appropriate.

In addition to the transactions that are excluded by the instructions to the SEC's related-person transaction disclosure rule, the Board of Directors has determined that the following transactions do not create a material direct or indirect interest on behalf of related persons and, therefore, are not related-person transactions for purposes of this policy:

interests arising solely from the related person's position as an executive officer of another entity (whether or not the person is also a director of such entity) that is a participant in the transaction, where (a) the related person and all other related persons own in the aggregate less than a 10% equity interest in such entity; (b) the related person and his or her immediate family members are not involved in the negotiation of the terms of the transaction and do not receive any special benefits as a result of the transaction and; (c) the amount involved in the transaction equals less than the greater of \$200,000 or 5% of the annual gross revenues of the company receiving payment under the transaction; and a transaction that is specifically contemplated by provisions of our certificate of incorporation or bylaws.

The policy provides that transactions involving compensation of executive officers shall be reviewed and approved by the compensation committee in the manner specified in its charter.

STOCKHOLDER PROPOSALS

Deadlines for stockholder proposals and director nominations

Stockholders who wish to present proposals for inclusion in our proxy materials for the annual meeting of stockholders to be held in 2017, or the 2017 Annual Meeting, may do so by following the procedures prescribed in Rule 14a-8 under the Securities Exchange Act of 1934 and in our Bylaws. Our Secretary must receive stockholder proposals intended to be included in our proxy statement and form of proxy relating to our 2017 Annual Meeting made under Rule 14a-8 by December 22, 2016.

Under our current Bylaws, proposals of business and nominations for directors other than those to be included in our proxy materials following the procedures described in Rule 14a-8 may be made by stockholders entitled to vote at the meeting if notice is timely given and if the notice contains the information required by the Bylaws. To be timely a notice with respect to the 2017 Annual Meeting must be delivered to our Secretary no earlier than February 1, 2017 and no later than March 3, 2017.

Any proposal of business or nomination should be mailed to: Joseph W. La Barge, Secretary, Spark Therapeutics, Inc. 3737 Market Street, Suite 1300, Philadelphia, PA 19104.

EXECUTIVE OFFICERS

The following table sets forth the name, age and position of each of our executive officers as of April 7, 2016.

NAME Age Position

Jeffrey D. Marrazzo (1) 37 Co-founder, Chief Executive Officer and Director

Katherine A. High, M.D. (2) 64 Co-founder, President, Chief Scientific Officer and Director

Rogério Vivaldi, M. D. 53 Chief Commercial Officer Stephen W. Webster 55 Chief Financial Officer

- (1)Mr. Marrazzo is also a director of the Company and his biographical information appears on page 5.
- (2) Dr. High is also a director of the Company and her biographical information appears on page 6.

Rogério Vivaldi, M.D. Dr. Vivaldi has served as our Chief Commercial Officer since December 2014. Prior to joining Spark, Dr. Vivaldi was Chief Executive Officer and President of Minerva Neurosciences, Inc. from November 2013 to December 2014. Prior to joining Minerva, Dr. Vivaldi served as Senior Vice President—Head of the Rare Diseases business unit at Genzyme Corporation, from October 2011 to October 2013. From July 2010 to September 2011, he was the Senior Vice President—Head of the Renal and Endocrinology business unit at Genzyme and from January 2004 to June 2010 he was the Senior Vice President—Head of Genzyme Latin America. Prior to 2004, Dr. Vivaldi founded Genzyme Brazil in 1997. Dr. Vivaldi served on our Board of Directors from April 2014 to December 2014 and served on the Board of Directors of Minerva Neurosciences from November 2013 to December 2014. Dr. Vivaldi holds a medical degree from the University of Rio de Janeiro and his M.B.A. from Federal University of Rio de Janeiro. Dr. Vivaldi completed his residency in metabolism

and endocrinology at Rio de Janeiro State University and his fellowship at Mount Sinai Hospital Center in New York, department of genetics, with an emphasis on Gaucher's disease.

Stephen W. Webster. Mr. Webster has served as our Chief Financial Officer since July 2014. From June 2012 to November 2013, he served as Senior Vice President, Finance and Chief Financial Officer at Optimer Pharmaceuticals. From June 2008 to December 2011, Mr. Webster served as Senior Vice President, Finance and Chief Financial Officer at Adolor Corporation. Mr. Webster has served on the Board of Directors of Viking Therapeutics Inc. since June 2014. Mr. Webster holds an A.B. in economics from Dartmouth College and an M.B.A. in finance from The Wharton School at the University of Pennsylvania.

Our executive officers are elected by, and serve at the discretion of, our Board of Directors.

EXECUTIVE COMPENSATION

This section describes the material elements of compensation awarded to, earned by or paid to our named executive officers. Our named executive officers for the year ended December 31, 2015 were Jeffrey D. Marrazzo, who serves as our Chief Executive Officer, Dr. Katherine A. High, M.D., who serves as our President and Chief Scientific Officer, and Stephen W. Webster, who serves as our Chief Financial Officer. This section also provides qualitative information regarding the manner and context in which compensation is awarded to and earned by our named executive officers and is intended to place in perspective the data presented in the tables and narrative that follow. Summary compensation table

The following table sets forth information regarding compensation awarded to, earned by or paid to our named executive officers during the year ended December 31, 2014 and 2015.

Name and principal position	Year Salary	Equity Awards (1)	Non-equity incentive plan compensation (2)	All other compensation (3)	Total
Jeffrey D. Marrazzo	2015 \$447,039(4)\$5,670,479	\$ 300,000	\$ 72,343	\$6,489,861
Chief Executive Officer	2014 \$349,604(5)\$1,128,401	\$ 146,301	\$ 40,509	\$1,664,815
Katherine A. High, M.D.	2015 \$403,558(6)\$3,175,603	\$ 230,000	\$ 17,835	\$3,826,996
President and Chief Scientific Officer	2014 \$108,538(7)\$408,701	\$ 45,896	\$ 8,169	\$571,304
Stephen W. Webster	2015 \$348,280(8)\$2,720,941	\$ 221,000	\$ 38,001	\$3,328,222
Chief Financial Officer	2014 \$147,528(9)\$481,494	\$ 52,500	\$ 12,861	\$694,383

The amounts reported in the "Equity awards" column reflect the aggregate fair value of share-based compensation awarded during the year computed in accordance with the provisions of Financial Accounting Standards Board

- (1) Accounting Standard Codification Topic 718, Compensation Stock Compensation. Note that the amounts reported in this column reflect the accounting cost for these stock options, and do not correspond to the actual economic value that may be received by the executives from the options.
 - The amounts reported in the "Non-equity incentive plan compensation" column represent signing bonuses as well as bonuses awarded in July 2014 to Mr. Marrazzo to reflect performance from July 1, 2013 to June 30, 2014, in
- (2) March 2015 to Messrs. Marrazzo and Webster for performance from July 1 to December 31, 2014 and in January 2016 for performance in 2015. Dr. High's 2014 bonus reflects the terms of our employment agreement with her and her 2015 bonus reflects a bonus award in January 2016 for performance in 2015.
- The compensation included in the "All other compensation" column consists of premiums we paid with respect to each of our named executive officers for: (a) medical, dental and vision insurance; (b) personal accident insurance;
- (c) life insurance; (d) long-term disability insurance; (e) short-term disability insurance; (f) matching contributions to our 401(k) plan; and (g) additional "safe harbor" contributions to our 401(k) plan for 2014 and 2015.

(4)

- In 2015 we paid an annualized base salary of \$392,000 to Mr. Marrazzo, which was increased to \$450,000 in February 2015.
- (5) In 2014, we paid an annualized base salary of \$300,000 to Mr. Marrazzo, which was increased to \$392,000 in June 2014.
- (6) $\frac{\text{In 2015}}{2015}$, we paid an annualized base salary of \$367,171 to Dr. High, which was increased to \$405,000 in February 2015.
- (7) Dr. High joined us on September 16, 2014. Dr. High's annualized base salary for 2014 was \$367,171.
- (8) In 2015, we paid an annualized base salary of \$300,000 to Mr. Webster, which was increased to \$350,000 in February 2015.
- (9)Mr. Webster joined us on July 7, 2014. Mr. Webster's annualized base salary for 2014 was \$300,000. Employment agreements with our named executive officers

We have written employment agreements with each of our named executive officers — Mr. Marrazzo, Dr. High and Mr. Webster. The agreements with each of Mr. Marrazzo, Dr. High and Mr. Webster provide for at-will employment. In addition, each of our named executive officers is subject to invention assignment, non-disclosure, non-competition and non-

solicitation agreements through separate agreements that were executed and delivered by the executives in connection with their employment agreements.

Pursuant to these agreements, each of our named executive officers received an annual base salary in 2015 as follows: Mr. Marrazzo: \$450,000; Dr. High: \$405,000; and Mr. Webster: \$350,000. In January 2016, the Compensation Committee approved the 2016 annual salaries for our named executive officers as follows: Mr. Marrazzo: \$525,000; Dr. High: \$415,000; and Mr. Webster: \$400,000.

Following the end of each calendar year, each named executive officer is eligible to receive an annual bonus based on the achievement of individual and company performance objectives. The amount of the annual bonus, if any, for Mr. Marrazzo will be determined by our Board of Directors in its sole discretion; the amount of the annual bonus, if any, for Dr. High and Mr. Webster will be determined by our Board of Directors and our Chief Executive Officer in their sole discretion. For each of our named executive officers, the bonus is calculated as a percentage of the named executive officer's annual base salary. For 2015, the target bonus percentages for each named executive officer was as follows: Mr. Marrazzo: 50%; Dr. High: 45%; and Mr. Webster: 40%. In January 2016, the Compensation Committee approved the 2016 target bonus percentages for each named executive officer as follows: Mr. Marrazzo: 60%; Dr. High: 45%; and Mr. Webster: 40%.

Potential payments upon termination or change in control

Pursuant to the terms of each executive officer's employment agreement, upon execution and effectiveness of a separation agreement and release of claims, each executive officer is entitled to severance payments if his or her employment is terminated under specified circumstances.

Mr. Marrazzo

If we terminate Mr. Marrazzo's employment without cause, or if Mr. Marrazzo terminates his employment with us for good reason, each as defined in his employment agreement, Mr. Marrazzo is entitled to continue receiving his base salary and insurance benefits for a period of 18 months following the date of termination of employment, the amount of any bonus determined by our Board of Directors to be payable to Mr. Marrazzo for the immediately preceding year that has not yet been paid and a payment in an amount equal to the pro rata portion of his target bonus for the fiscal year in which the termination occurs.

If, within 24 months following a change in control, as defined in Mr. Marrazzo's employment agreement, we terminate Mr. Marrazzo's employment without cause or Mr. Marrazzo terminates his or her employment with us for good reason, Mr. Marrazzo is entitled to continue receiving his base salary and insurance benefits for a period of 24 months following the date of termination of employment, the amount of any bonus determined by our Board of Directors to be payable to him for the immediately preceding year that has not yet been paid, a payment in an amount equal to the pro rata portion of Mr. Marrazzo's target bonus for the fiscal year in which the termination occurs and an additional payment equal to 2 times his or her target bonus for the fiscal year in which the termination occurs.

Other executive officers

If we terminate any of our other executive officers' employment without cause, or if such executive officer terminates his or her employment with us for good reason, each as defined in his or her employment agreement, such executive officer is entitled to continue receiving his or her base salary and insurance benefits for a period of 12 months following the date of termination of employment, the amount of any bonus determined by our Board of Directors to be payable to the executive offericer for the immediately preceding year that has not yet been paid and a payment in an amount equal to the pro rata portion of his or her target bonus for the fiscal year in which the termination occurs. If, within 24 months following a change in control, as defined in such executive officer's employment agreement, we terminate such executive officer's employment without cause or such executive officer terminates his or her employment with us for good reason, such executive officer is entitled to continue receiving his or her base salary and insurance benefits for a period of 18 months following the date of termination of employment, the amount of any bonus determined by our Board of Directors to be payable to the executive for the immediately preceding year that has not yet been paid, a payment in an amount equal to the pro rata portion of such executive officer's target bonus for the fiscal year in which the termination occurs and an additional payment equal to 1.5 times his or her target bonus for the fiscal year in which the termination occurs.

Equity awards

If we terminate any of our executive officers' employment without cause, or if any such executive officer terminates his or her employment with us for good reason, such named executive officer's unvested equity awards will vest on a monthly basis for the period from the last vesting date of each equity award through the date of termination of his or her employment.

In addition, if there occurs a change of control, any unvested equity granted prior to the corporate conversion will become vested. Additionally, 50% of the unvested portion of each of our executive officers' unvested equity awards that were

outstanding at the time of the change of control will vest immediately, and the remaining 50% will vest in equal quarterly installments over the following two years or, if shorter, over the remaining period of the award's original vesting schedule; provided, however, that the new vesting schedule will not replace any more favorable vesting acceleration provision provided for in any equity award agreement governing an equity award held by such executive officer. Following a termination without cause or for good reason within 24 months of the change of control, such executive officer's unvested equity awards that were outstanding at the time of the change of control will vest in full. To the extent that any severance or other compensation payment to any of our executive officers pursuant to an employment agreement or any other agreement constitutes an "excess parachute payment" within the meaning of Sections 280G and 4999 of the Internal Revenue Code of 1986, as amended, then such executive officer will receive the full amount of such severance and other payments, or a reduced amount intended to avoid the application of Sections 280G and 4999, whichever provides the executive with the highest amount on an after-tax basis. Limitation of liability and indemnification

Our Restated Certificate of Incorporation limits the personal liability of directors for breach of fiduciary duty to the maximum extent permitted by the Delaware General Corporation Law, or DGCL, and provides that no director will have personal liability to us or to our stockholders for monetary damages for breach of fiduciary duty or other duty as a director. However, these provisions do not eliminate or limit the liability of any of our directors:

for any breach of the director's duty of loyalty to us or our stockholders;

for acts or omissions not in good faith or which involve intentional misconduct or a knowing violation of law; for voting or assenting to unlawful payments of dividends, stock repurchases or other distributions; or for any transaction from which the director derived an improper personal benefit.

Any amendment to, or repeal of, these provisions will not eliminate or reduce the effect of these provisions in respect of any act, omission or claim that occurred or arose prior to such amendment or repeal. If the DGCL is amended to provide for further limitations on the personal liability of directors of corporations, then the personal liability of our directors will be further limited to the greatest extent permitted by the DGCL.

In addition, our Restated Certificate of Incorporation provides that we must indemnify our directors and officers and we must advance expenses, including attorneys' fees, to our directors and officers in connection with legal proceedings, subject to very limited exceptions.

We maintain a general liability insurance policy that covers certain liabilities of our directors and executive officers arising out of claims based on acts or omissions in their capacities as directors or executive officers. In addition, we intend to enter into indemnification agreements with each of our directors and executive officers. These indemnification agreements may require us, among other things, to indemnify each such director or executive officer for some expenses, including attorneys' fees, judgments, fines and settlement amounts incurred by him or her in any action or proceeding arising out of his or her service as one of our directors or executive officers.

Certain of our non-employee directors may, through their relationships with their employers, be insured and/or indemnified against certain liabilities incurred in their capacity as members of our Board of Directors.

Insofar as indemnification for liabilities arising under the Securities Act of 1933, as amended, or the Securities Act, may be permitted to directors, executive officers or persons controlling us, in the opinion of the SEC, such indemnification is against public policy as expressed in the Securities Act and is therefore unenforceable. Rule 10b5-1 plans

Certain of our executive officers have adopted, and other executive officers may in the future adopt, written plans, known as Rule 10b5-1 plans, in which they will contract with a broker to buy or sell shares of our common stock on a periodic basis. Under a Rule 10b5-1 plan, a broker executes trades pursuant to parameters established by the officer when entering into the plan, without further direction from the director or officer. It also is possible that the officer could amend or terminate the plan when not in possession of material, nonpublic information. In addition, our executive officers may buy or sell additional shares outside of a Rule 10b5-1 plan when they are not in possession of material, nonpublic information.

Equity incentive awards

Although we do not have a formal policy with respect to the grant of equity incentive awards to our named executive officers, or any formal equity ownership guidelines applicable to them, we believe that equity grants provide our executives with a strong link to our long-term performance, create an ownership culture and help to align the interests of our executives and our stockholders. In addition, we believe that equity grants with a time-based vesting feature promote executive retention because this feature incentivizes our executives to remain in our employment during the vesting period. Accordingly, our Board of

Directors periodically reviews the equity incentive compensation of our executive officers and, from time to time, may grant equity incentive awards to them in the form of stock options or other equity awards.

2015 outstanding option awards at fiscal year-end

Option Awards

The following table sets forth information concerning outstanding equity awards at December 31, 2015 for each of our named executive officers.

Stock Awards

	Option / twarts				Stock / Iwards		
Name	options	securities	Option exercise price (\$/share)	Option expiration date	Number of shares or units of stock that have not vested (#)	stock that	
Jeffery D. Marrazzo (2)	_	125,000	\$ 68.45	3/15/2025		_	
	163,902	273,169	\$ 3.45	7/2/2024	_		
Katherine A. High, M.D. (3)		70,000	\$ 68.45	3/15/2025	125,000	\$5,663,750	
	59,343	98,906	\$ 3.45	7/2/2024			
Stephen W. Webster (4)		60,000	\$ 68.45	3/15/2025			
	38,130	127,885	\$ 3.45	7/7/2024			

- (1) The fair market value of the unvested stock awards was calculated using our common stock price of \$45.31 per share, the closing price of our common stock on the Nasdaq Global Select Market on December 31, 2015.

 Mr. Marrazzo's option to purchase 437,071 shares of common stock vests as follows: 25% vested on May 24, 2015.
- (2) and the remainder vests in equal quarterly installments over the following three years. Mr. Marrazzo's option to purchase 125,000 shares of common stock vests as follows: 25% vests on March 16, 2016 and the remainder vests in equal quarterly installments over the following three years.
 - Dr. High's restricted stock vests as follows: 25% of the shares vested on March 13, 2014 with the remaining shares vesting in equal quarterly installments over the following three years. Dr. High's option to purchase 158,249 shares
- (3) of common stock vests as follows: 25% vested on May 24, 2015 and the remainder vests in equal quarterly installments over the following three years. Dr. High's option to purchase 70,000 shares of common stock vests as follows: 25% vests on March 16, 2016 and the remainder vests in equal quarterly installments over the following three years.
- Mr. Webster's option to purchase 186,015 shares of common stock vests as follows: 25% vested on July 7, 2015 and the remainder vests in equal quarterly installments over the following three years. Mr. Webster's option to purchase 60,000 shares of common stock vests as follows: 25% vests on March 16, 2016 and the remainder vests in equal quarterly installments over the following three years.

EQUITY INCENTIVE AND OTHER COMPENSATION PLANS

Stock option and other compensation plans

In this section we describe our 2014 stock incentive plan, as amended to date, or the 2014 plan, our 2015 stock incentive plan, or the 2015 plan, and our 2015 employee stock purchase plan. Prior to our initial public offering, we granted awards to eligible participants under the 2014 plan. Following the effectiveness of the registration statement for our initial public offering, we ceased granting awards under the 2014 plan and started granting awards to eligible participants under the 2015 plan.

2014 plan

The 2014 plan was adopted by our Board of Directors and approved by our stockholders in May 2014. The 2014 plan provides for the grant of incentive stock options, nonstatutory stock options, restricted stock awards, restricted stock

unit awards, stock appreciation rights and other stock-based awards. Our employees, officers, directors, consultants and advisors are eligible to receive awards under our 2014 plan; however, incentive stock options may only be granted to our employees.

The type of award granted under our 2014 plan and the terms of such award are set forth in the applicable award agreement.

Pursuant to the terms of the 2014 plan, our Board of Directors (or a committee delegated by our Board of Directors) administers the plan and, subject to any limitations in the plan, selects the recipients of awards and determines:

the number of shares of our common stock covered by options and the dates upon which the options become exercisable;

the type of options to be granted;

the duration of options, which may not be in excess of ten years;

the exercise price of options, which must be at least equal to the fair market value of our common stock on the date of grant; and

the number of shares of our common stock subject to, and the terms of any stock appreciation rights, restricted stock awards, restricted stock units or other stock-based awards and the terms and conditions of such awards, including conditions for repurchase, measurement price, issue price and repurchase price (though the measurement price of stock appreciation rights must be at least equal to the fair market value of our common stock on the date of grant and the duration of such awards may not be in excess of ten years).

Effect of certain changes in capitalization.

Upon the occurrence of any of a stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event, or any dividend or distribution to holders of our common stock other than an ordinary cash dividend, our Board of Directors shall equitably adjust:

the number and class of securities available under the 2014 plan;

the number and class of securities and exercise price per share of each outstanding option;

the share and per-share provisions and the measurement price of each outstanding stock appreciation right; the number of shares subject to, and the repurchase price per share subject to, each outstanding restricted stock award; and

the share and per-share related provisions and the purchase price, if any, of each other stock-based award. Effect of certain corporate transactions

Upon a merger or other reorganization event (as defined in our 2014 plan), our Board of Directors may, in its sole discretion, take any one or more of the following actions pursuant to the 2014 plan as to some or all outstanding awards other than restricted stock:

provide that all outstanding awards shall be assumed, or substantially equivalent awards shall be substituted, by the acquiring or successor corporation (or an affiliate thereof);

upon written notice to a participant, provide that all of the participant's vested but unexercised awards will terminate immediately prior to the consummation of such reorganization event unless exercised by the participant; provide that outstanding awards shall become exercisable, realizable or deliverable, or restrictions applicable to an award shall lapse, in whole or in part, prior to or upon such reorganization event;

in the event of a reorganization event pursuant to which holders of shares of our common stock will receive a cash payment for each share surrendered in the reorganization event, make or provide for a cash payment to the participants with respect to each award held by a participant equal to (1) the number of shares of our common stock subject to the vested portion of the award (after giving effect to any acceleration of vesting that occurs upon or immediately prior to such reorganization event) multiplied by (2) the excess, if any, of the cash payment for each share surrendered in the reorganization event over the exercise, measurement or purchase price of such award and any applicable tax withholdings, in exchange for the termination of such award; and/or

provide that, in connection with a liquidation or dissolution, awards shall convert into the right to receive liquidation proceeds (if applicable, net of the exercise, measurement or purchase price thereof and any applicable tax withholdings).

Our Board of Directors does not need to take the same action with respect to all awards and may take different actions with respect to portions of the same award.

In the case of certain restricted stock units, no assumption or substitution is permitted, and the restricted stock units will instead be settled in accordance with the terms of the applicable restricted stock unit agreement.

Upon the occurrence of a reorganization event other than a liquidation or dissolution, the repurchase and other rights with respect to outstanding awards of restricted stock will continue for the benefit of the successor company and will, unless the Board of Directors may otherwise determine, apply to the cash, securities or other property into which shares of our common stock are converted or exchanged pursuant to the reorganization event. Upon the occurrence of a reorganization event involving a liquidation or dissolution, all restrictions and conditions on each outstanding restricted stock award will automatically be deemed terminated or satisfied, unless otherwise provided in the agreement evidencing the restricted stock award.

At any time, our Board of Directors may, in its sole discretion, provide that any award under the 2014 plan will become immediately exercisable in full or in part, free of some or all restrictions or conditions, or otherwise realizable in full or in part.

As of April 7, 2016, there were options to purchase 2,036,803 shares of our common stock outstanding under the 2014 plan, at a weighted-average exercise price of \$4.36 per share, and options to purchase 235,659 shares of our common stock had been exercised.

Our Board of Directors may amend, suspend or terminate the 2014 plan at any time, except that stockholder approval may be required to comply with applicable law or stock market requirements.

2015 plan

Our 2015 plan, which became effective on January 30, 2015, was adopted by our Board of Directors and approved by our stockholders in January 2015. The 2015 plan provides for the grant of incentive stock options, nonstatutory stock options, stock appreciation rights, restricted stock awards, restricted stock units and other stock-based awards. Under the 2015 plan, the number of shares of our common stock that is reserved for issuance under the 2015 plan is the sum of: (1) 1,830,000 plus; (2) 209,519 shares reserved for issuance under the 2014 plan that remained available for grant under the 2014 plan immediately prior to the closing of our initial public offering and the number of shares of our common stock subject to outstanding awards under the 2014 plan that expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by us at their original issuance price pursuant to a contractual repurchase right; plus (3) an annual increase, to be added on the first day of each fiscal year, beginning with the fiscal year ending December 31, 2016 and continuing until, and including, the fiscal year ending December 31, 2025, equal to the lowest of 1,724,000 shares of our common stock, 4% of the number of shares of our common stock outstanding on the first day of such fiscal year and an amount determined by our Board of Directors. As of January 1, 2016 the number of shares authorized for issuance under the 2015 plan automatically increased, pursuant to the terms of the plan, by 1,083,313 shares of common stock.

Our employees, officers, directors, consultants and advisors are eligible to receive awards under the 2015 plan. Incentive stock options, however, may only be granted to our employees.

Pursuant to the terms of the 2015 plan, our Board of Directors (or a committee delegated by our Board of Directors) administer the plan and, subject to any limitations in the plan, will select the recipients of awards and determine:

the number of shares of our common stock covered by options and the dates upon which the options become exercisable;

the type of options to be granted;

the duration of options, which may not be in excess of ten years;

the exercise price of options, which must be at least equal to the fair market value of our common stock on the date of grant; and

the number of shares of our common stock subject to and the terms of any stock appreciation rights, restricted stock awards, restricted stock units or other stock-based awards and the terms and conditions of such awards, including conditions for repurchase, issue price and repurchase price (though the measurement price of stock appreciation rights must be at least equal to the fair market value of our common stock on the date of grant and the duration of such awards may not be in excess of ten years).

If our Board of Directors delegates authority to an executive officer to grant awards under the 2015 plan, the executive officer will have the power to make awards to all of our employees, except executive officers. Our Board of Directors will fix the terms of the awards to be granted by such executive officer, including the exercise price of such awards (which may include a formula by which the exercise price will be determined), and the maximum number of shares subject to awards that such executive officer may make.

As of April 7, 2016, there were options to purchase 2,017,750 shares of our common stock outstanding under the 2015 plan, at a weighted-average exercise price of \$49.52 per share, and no options to purchase shares of our common stock had been exercised.

Effect of certain changes in capitalization

Upon the occurrence of any stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event, or any dividend or distribution to holders of our common stock other than an ordinary cash dividend, our Board of Directors shall equitably adjust:

the number and class of securities available under the 2015 plan;

the share counting rules under the 2015 plan;

the number and class of securities and exercise price per share of each outstanding option;

the share and per-share provisions and the measurement price of each outstanding stock appreciation right; the number of shares subject to, and the repurchase price per share subject to, each outstanding restricted stock award; and

the share and per-share related provisions and the purchase price, if any, of each other stock-based award.

Effect of certain corporate transactions

Upon a merger or other reorganization event (as defined in our 2015 plan), our Board of Directors may, on such terms as our board determines (except to the extent specifically provided otherwise in an applicable award agreement or other agreement between the participant and us), take any one or more of the following actions pursuant to the 2015 plan as to some or all outstanding awards, other than restricted stock awards:

provide that all outstanding awards shall be assumed, or substantially equivalent awards shall be substituted, by the acquiring or successor corporation (or an affiliate thereof);

upon written notice to a participant, provide that all of the participant's unvested and/or vested but unexercised awards will terminate immediately prior to the consummation of such reorganization event unless exercised by the participant (to the extent then exercisable);

provide that outstanding awards shall become exercisable, realizable or deliverable, or restrictions applicable to an award shall lapse, in whole or in part, prior to or upon such reorganization event;

in the event of a reorganization event pursuant to which holders of shares of our common stock will receive a cash payment for each share surrendered in the reorganization event, make or provide for a cash payment to the participants with respect to each award held by a participant equal to (1) the number of shares of our common stock subject to the vested portion of the award (after giving effect to any acceleration of vesting that occurs upon or immediately prior to such reorganization event) multiplied by (2) the excess, if any, of the cash payment for each share surrendered in the reorganization event over the exercise, measurement or purchase price of such award and any applicable tax withholdings, in exchange for the termination of such award; and/or

provide that, in connection with a liquidation or dissolution, awards shall convert into the right to receive liquidation proceeds (if applicable, net of the exercise, measurement or purchase price thereof and any applicable tax withholdings).

Our Board of Directors does not need to take the same action with respect to all awards, all awards held by a participant or all awards of the same type.

In the case of certain restricted stock units, no assumption or substitution is permitted, and the restricted stock units will instead be settled in accordance with the terms of the applicable restricted stock unit agreement. Upon the occurrence of a reorganization event other than a liquidation or dissolution, the repurchase and other rights with respect to outstanding restricted stock awards will continue for the benefit of the successor company and will, unless the Board of Directors may otherwise determine, apply to the cash, securities or other property into which shares of our common stock are converted or exchanged pursuant to the reorganization event. Upon the occurrence of a reorganization event involving a liquidation or dissolution, all restrictions and conditions on each outstanding restricted stock award will automatically be deemed terminated or satisfied, unless otherwise provided in the agreement evidencing the restricted stock award or any other agreement between the participant and us. The 2015 plan provides that, except to the extent specifically provided to the contrary in an award agreement or any other agreement between the participant and us, immediately prior to a change in control event (as defined in our 2015 plan), the vesting schedule of each outstanding option and restricted stock award shall be accelerated in part so that 50% of the unvested portion of such award shall immediately become exercisable or free from forfeiture or repurchase, as applicable, and the remaining 50% shall vest in substantially equal quarterly installments over the following two years, or, if shorter, in accordance with the original vesting schedule set forth in the award agreement governing such award. Additionally, each such option or restricted stock award shall vest in full and become exercisable or free from forfeiture or repurchase, as applicable, if, on or prior to the second anniversary of the change in control event, the participant's employment with the company or the acquiring company is terminated for good reason by the participant or is terminated without cause by the company or the acquiring corporation (as such terms are defined in the 2015 plan). However, if the acquiring corporation does not provide for the assumption or

substitution of unvested options or restricted stock awards in connection with the change in control event, each such option and restricted stock award shall vest in full and become exercisable or free from forfeiture or repurchase, as applicable, immediately prior to the change in control event. Our Board of Directors may specify in an award agreement at the time of grant the effect of a change in control event on an restricted stock units, stock appreciation

rights or other stock-based awards.

At any time, our Board of Directors may, in its sole discretion, provide that any award under the 2015 plan will become immediately exercisable in full or in part, free of some or all restrictions or conditions, or otherwise realizable in whole or in part as the case may be.

No award may be granted under the 2015 plan on or after January 29, 2025. Our Board of Directors may amend, suspend or terminate the 2015 plan at any time, except that stockholder approval may be required to comply with applicable law or stock market requirements.

2015 employee stock purchase plan

Our 2015 employee stock purchase plan, or the 2015 ESPP, which became effective upon the closing of our initial public offering, was adopted by our Board of Directors and approved by our stockholders in January 2015. The 2015 ESPP is administered by our Board of Directors or by a committee appointed by our Board of Directors. Upon implementation, the 2015 ESPP will provide participating employees with the opportunity to purchase an aggregate of 220,000 shares of our common stock. The number of shares of our common stock reserved for issuance under the 2015 ESPP automatically will increase on the first day of each fiscal year, beginning with the fiscal year ending December 31, 2016 and continuing until, and including, the fiscal year ending December 31, 2026, in an amount equal to the lowest of: (1) 440,000 shares of our common stock; (2) 1% of the total number of shares of our common stock outstanding on the first day of the applicable fiscal year; and (3) an amount determined by our Board of Directors. As of January 1, 2016, the number of shares authorized for issuance under the 2015 ESPP automatically increased, pursuant to the terms of the plan, by 270,828 shares of common stock.

All of our employees and employees of any of our designated subsidiaries, as defined in the 2015 ESPP, are eligible to participate in the 2015 ESPP, provided that:

such person is customarily employed by us or a designated subsidiary for more than 20 hours a week and for more than five months in a calendar year; and

such person was our employee or an employee of a designated subsidiary at least fifteen business days prior to the first day of the applicable offering period under the 2015 ESPP.

No employee may purchase shares of our common stock under the 2015 ESPP and any of our other employee stock purchase plans in excess of \$25,000 of the fair market value of our common stock (as of the date of the option grant) in any calendar year. In addition, no employee may purchase shares of our common stock under the 2015 ESPP that would result in the employee owning 5% or more of the total combined voting power or value of our stock. We expect to make one or more offerings to our eligible employees to purchase stock under the 2015 ESPP beginning at such time as our Board of Directors may determine. Each offering will consist of a six-month offering period during which payroll deductions will be made and held for the purchase of our common stock at the end of the offering period. Our Board of Directors may, at its discretion, choose a different period of not more than 12 months for offerings.

On the commencement date of each offering period, each eligible employee may authorize up to a maximum of 15% of his or her compensation to be deducted by us during the offering period. Each employee who continues to be a participant in the 2015 ESPP on the last business day of the offering period will be deemed to have exercised an option to purchase from us the number of whole shares of our common stock that his or her accumulated payroll deductions on such date will buy, not in excess of the maximum numbers set forth above. Under the terms of the 2015 ESPP, the purchase price shall be determined by our Board of Directors for each offering period and will be at least 85% of the applicable closing price of our common stock. If our Board of Directors does not make a determination of the purchase price, the purchase price will be 85% of the lesser of the closing price of our common stock on the first business day of the offering period or on the last business day of the offering period.

An employee may for any reason withdraw from participation in an offering prior to the end of an offering period and permanently withdraw the balance accumulated in the employee's account. If an employee elects to discontinue his or her payroll deductions during an offering period but does not elect to withdraw his or her funds, funds previously deducted will be applied to the purchase of common stock at the end of the offering period. If a participating employee's employment ends before the last business day of an offering period, no additional payroll deductions will be made and the balance in the employee's account will be paid to the employee.

We will be required to make equitable adjustments to the number and class of securities available under the 2015 ESPP, the share limitations under the 2015 ESPP and the purchase price for an offering period under the 2015 ESPP to reflect stock splits, reverse stock splits, stock dividends, recapitalizations, combinations of shares, reclassifications of shares, spin-offs and other similar changes in capitalization or events or any dividends or distributions to holders of our common stock other than ordinary cash dividends.

In connection with a merger or other reorganization event (as defined in the 2015 ESPP), our Board of Directors or a committee of our Board of Directors may take any one or more of the following actions as to outstanding options to purchase shares of our common stock under the 2015 ESPP on such terms as our board or committee determines:

provide that options shall be assumed, or substantially equivalent options shall be substituted, by the acquiring or succeeding corporation (or an affiliate thereof);

upon written notice to employees, provide that all outstanding options will be terminated immediately prior to the consummation of such reorganization event and that all such outstanding options will become exercisable to the extent

of accumulated payroll deductions as of a date specified by our board or committee in such notice, which date shall not be less than ten days preceding the effective date of the reorganization event;

upon written notice to employees, provide that all outstanding options will be cancelled as of a date prior to the effective date of the reorganization event and that all accumulated payroll deductions will be returned to participating employees on such date;

in the event of a reorganization event under the terms of which holders of our common stock will receive upon consummation thereof a cash payment for each share surrendered in the reorganization event, change the last day of the offering period to be the date of the consummation of the reorganization event and make or provide for a cash payment to each employee equal to (1) the cash payment for each share surrendered in the reorganization event times the number of shares of our common stock that the employee's accumulated payroll deductions as of immediately prior to the reorganization event could purchase at the applicable purchase price, where the acquisition price is treated as the fair market value of our common stock on the last day of the applicable offering period for purposes of determining the purchase price and where the number of shares that could be purchased is subject to the applicable limitations under the 2015 ESPP minus (2) the result of multiplying such number of shares by the purchase price; and/or

provide that, in connection with our liquidation or dissolution, options shall convert into the right to receive liquidation proceeds (net of the purchase price thereof).

The 2015 ESPP may be terminated at any time by our Board of Directors. Upon termination, we will refund all amounts in the accounts of participating employees.

401(k) retirement plan

We maintain a 401(k) retirement plan that is intended to be a tax-qualified defined contribution plan under Section 401(k) of the Internal Revenue Code. In general, all of our employees are eligible to participate, beginning on the first day of the month following commencement of their employment. The 401(k) plan includes a salary deferral arrangement pursuant to which participants may elect to reduce their current compensation by up to the statutorily prescribed limit, equal to \$18,000 in 2015, and have the amount of the reduction contributed to the 401(k) plan. Participants who are at least 50 years old also can make "catch-up" contributions, which in 2015 may be up to an additional \$5,500 above the statutory limit. We also currently make discretionary matching contributions to our 401(k) plan equal to 50% of the employee contributions up to 6% of the employee's salary, subject to the statutorily prescribed limit, equal to \$18,000 in 2015. The match immediately vests in full. In addition to the discretionary matching contributions, we make a safe harbor contribution in an amount equal to 3% of your eligible compensation, subject to a maximum compensation of \$265,000, which immediately vests in full.

Securities authorized for issuance under equity compensation plans

The following table contains information about our equity compensation plans as of December 31, 2015. As of December 31, 2015, we had three equity compensation plans, our 2015 plan, 2015 ESPP, and 2014 plan, which was approved by our stockholders.

	Number of securities Number of securities to Weighted-average exercise remaining available for			
Plan category	be issued upon exercise of outstanding options, warrants and rights	1	future issuance under equity compensation plans (excluding securities	
	warrants and rights	rigitis	reflected in column(a))	
	(a)	(b)	(c)	
Equity compensation plans approved by security holders	3,071,372	\$22.99	1,190,275	
Equity compensation plans not approved by security holders	_	_	_	

Total 3,071,372 \$22.99 1,190,275

As described above in connection with the closing of our initial public offering, our Board of Directors and stockholders approved two new equity compensation plans. The 2015 plan initially provided for the reservation of 2,039,500 shares of common stock (including the 209,500 shares remaining under our 2014 plan and an additional 1,830,000 shares) for equity awards and includes provisions for an annual increase, to be added the first day of each fiscal year, beginning with the fiscal year ending December 31, 2016 and continuing until, and including, the fiscal year ending December 31, 2025, with such annual increase to be equal to the lowest of 1,724,000 shares of our common stock, 4% of the number of shares of our common

stock outstanding on the first day of the fiscal year and an amount determined by our Board of Directors and, as of January 1, 2016, the number of shares authorized for issuance under the 2015 plan automatically increased, pursuant to these terms by 1,083,313 shares of common stock. The 2015 ESPP initially provided for the reservation of 220,000 shares of common stock for purchase by participating employees and includes provisions for an annual increase, to be added the first day of each fiscal year, beginning with the fiscal year ending December 31, 2016 and continuing until, and including, the fiscal year ending December 31, 2026, with such annual increase to be equal to the lowest of 440,000 shares of our common stock, 1% of the number of shares of our common stock outstanding on the first day of the fiscal year and an amount determined by our Board of Directors and, as of January 1, 2016, the number of shares authorized for issuance under the 2015 ESPP automatically increased, pursuant to these terms by 270,828 shares of common stock.

RISK CONSIDERATION IN OUR COMPENSATION PROGRAM

Limits on hedging and pledging

As part of our insider trading policy, all employees, including executive officers, and members of our Board of Directors are prohibited from engaging in certain types of hedging transactions involving our securities, specifically short sales, including short sales "against the box," and purchases or sales of puts, calls or other derivative securities. Our insider trading policy also prohibits certain types of pledges of our securities by all employees, including executive officers, and members of our Board of Directors, specifically purchases of our securities on margin, borrowing against our securities held in a margin account or pledging our securities as collateral for a loan, with an exception for pledges of our securities as collateral for a loan only after certain prerequisites are met and only with the pre-approval of our Chief Financial Officer or General Counsel.

Tax and accounting considerations

Section 162(m) of the Code, as amended, generally disallows a tax deduction for compensation in excess of \$1.0 million paid in any year to certain of our officers. Qualifying performance-based compensation is not subject to the deduction limitation if specified requirements are met. We intend to periodically review the potential consequences of Section 162(m), and we generally intend to structure the performance-based portion of our executive compensation, where feasible, to comply with exemptions in Section 162(m) so that the compensation will remain tax deductible to us. However, the compensation committee may, in its judgment, authorize compensation payments that do not comply with the exemptions in Section 162(m) when it believes that such payments are appropriate to attract and retain executive talent and are in the best interests of our stockholders.

We account for equity compensation paid to our employees in accordance with FASB Accounting Standards Codification Topic 718, Compensation-Stock Compensation, or ASC 718, which requires us to measure and recognize compensation expense in our financial statements for all stock-based payments based on an estimate of their fair value over the service period of the award. We record cash compensation as an expense at the time the obligation is accrued. DIRECTOR COMPENSATION

The following table sets forth a summary of the compensation we paid to our non-employee directors during 2015. Neither Mr. Marrazzo nor Dr. High, our directors who also serve as our Chief Executive Officer and our President and Chief Scientific Officer, respectively, receive any additional compensation for his or her service as a director. The compensation received by Mr. Marrazzo and Dr. High as an employee during 2015 is presented in "Summary Compensation Table".

1			
Name	Fees Earned or paid in cash (\$)	Option awards (\$) (4)	Total (\$)
Steven Altschuler, M.D. (1)	\$57,326	_	\$57,326
A. Lorris Betz, M.D., Ph.D. (2)	\$31,597	\$412,756	\$444,353
Lars Ekman, M.D., Ph.D.	\$52,083	_	\$52,083
Anand Mehra, M.D.	\$47,396	_	\$47,396
Vincent J. Milano	\$56,597	_	\$56,597
Elliott Sigal, M.D., Ph.D.	\$43,785	_	\$43,785
Lota Zoth, CPA (3)	_		

1. Prior to June 30, 2015, all payments that Dr. Altschuler received in cash were paid directly to CHOP Foundation.

All payments that Dr. Betz received in cash are paid directly to the CHOP Foundation. On January 30, 2015, Dr. Betz received an option to purchase 27,000 shares of our common stock with an exercise price equal to \$23.00 per share (equal to the initial public offering price of our shares). Subject to Dr. Betz's continued service on our board, this stock option will vest in equal quarterly installments over the following three years. Any economic value received from such options will be re-mitted to the CHOP Foundation.

- 3. Ms. Zoth was appointed to our Board of Directors on January 6, 2016, therefore no fees were earned or options granted to her in 2015.
 - The amounts reported in the Option awards column represent the grant date fair value of the stock options granted to our non-employee directors during 2015 as computed in accordance with Accounting Standards Codification, or ASC, Topic 718 Compensation Stock Compensation, not including any estimates of forfeitures. See note 8 of
- 4. "Notes to Consolidated Financial Statements" in our Annual Report on Form 10-K filed with the SEC on March 14, 2016 for a discussion of assumptions made by the Company in determining the grant date fair value of our Option awards for the fiscal year ended December 31, 2015. Note that the amounts reported in this column reflect the accounting cost for these stock options, and do not correspond to the actual economic value that may be received by the directors from the options.

Director compensation arrangements

Since our initial public offering, our non-employee directors are compensated for their services on our Board of Directors as follows:

- each non-employee director receives an annual retainer of \$35,000;
- the chairman of the board receives an additional annual retainer of \$25,000; and
- each non-employee director who serves as member of a committee of our Board of Directors receives additional compensation as follows:
- audit committee—an annual retainer of \$7,500; chair an annual retainer of \$15,000;
- compensation committee—an annual retainer of \$5,000; chair an annual retainer of \$10,000; and
- nominating and corporate governance committee—an annual retainer of \$3,500; chair an annual retainer of \$7,500; and science and technology committee—an annual retainer of \$5,000; if a non-director chair, an annual retainer of \$10,000. Each annual retainer is payable in arrears in four equal quarterly installments on the last day of each quarter, provided that the amount of each payment will be prorated for any portion of a quarter that a director is not serving on our board.

Each member of our Board of Directors also will continue to be entitled to be reimbursed for reasonable travel and other expenses incurred in connection with attending meetings of the Board of Directors and any committee of the Board of Directors on which he or she serves.

SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS, MANAGEMENT AND RELATED STOCKHOLDER MATTERS

The following table sets forth information with respect to the beneficial ownership of our common stock, as of April 7, 2016 by:

each person known by us to beneficially own more than 5% of our common stock;

each of our directors:

each of our named executive officers;

and all of our executive officers and directors as a group.

The number of shares beneficially owned by each stockholder is determined under rules issued by the SEC and includes voting or investment power with respect to securities. Under these rules, beneficial ownership includes any shares as to which the individual or entity has sole or shared voting power or investment power. Each of the stockholders listed has sole voting and investment power with respect to the shares beneficially owned by the stockholder unless noted otherwise, subject to community property laws where applicable. The information is not necessarily indicative of beneficial ownership for any other purpose.

The percentage ownership calculations for beneficial ownership are based on 27,379,787 shares of common stock outstanding as of April 7, 2016. In computing the number of shares beneficially owned by an individual or entity and the percentage ownership of that person, shares of common stock subject to options, warrants or other rights held by such person that are currently exercisable, or will become exercisable within 60 days of April 7, 2016, are considered outstanding, although these shares are not considered outstanding for purposes of computing the percentage ownership of any other person.

Unless otherwise indicated, the address of all listed stockholders is 3737 Market Street, Suite 1300, Philadelphia, PA 19104.

Name of Beneficial Owner	Number of shares beneficially owned	Percentage of shares beneficially owned	
Named Executive Officers and Directors			
Jeffrey D. Marrazzo(1)	452,469	1.7	%
Katherine A. High, M.D.(2)	376,734	1.3	%
Stephen W. Webster(3)	78,881	*	
Steven Altschuler, M.D.(4)	21,276	*	
A. Lorris Betz, M.D., Ph.D.(5)	11,250	*	
Lars Ekman, M.D., Ph.D.(6)	16,276	*	
Anand Mehra, M.D.(7)	1,582,347	5.8	%
Vincent J. Milano(8)	16,276	*	
Elliott Sigal, M.D., Ph.D.(9)	63,192	*	
All executive officers and directors as a group (10 persons)	2,665,405	9.6	%
5% Stockholders			
CHOP Foundation(10)	7,794,905	28.4	%
FMR LLC (11)	4,019,544	14.7	%
T. Rowe Price Associates, Inc. (12)	2,052,928	7.5	%
Sofinnova Venture Partners VIII, L.P.(13) * Less than 1%	1,580,022	5.8	%

Consists of (a) 230,000 shares of common stock and (b) 222,469 shares of shares of common stock underlying options that are exercisable as of April 7, 2016 or will become exercisable within 60 days after such date owned by Mr. Marrazzo.

- Consists of (a) 290,000 shares of common stock and (b) 86,734 shares of shares of common stock underlying
- (2) options that are exercisable as of April 7, 2016 or will become exercisable within 60 days after such date owned by Dr. High.
 - Consists of (a) 2,500 shares of common stock and (b) 76,381 shares of shares of common stock underlying options
- (3) that are exercisable as of April 7, 2016 or will become exercisable within 60 days after such date owned by Mr. Webster.
 - Consists of (a) 5,000 shares of common stock owned by Dr. Altschuler and (b) 16,276 shares of common stock
- (4) underlying options that are exercisable as of April 7, 2016 or will become exercisable within 60 days after such date that were issued to Dr. Altschuler in his previous capacity as chief executive

officer of the CHOP Foundation. Dr. Altschuler holds such options as a nominee of the CHOP Foundation, which shall receive any economic benefit associated with such option. Dr. Altschuler disclaims beneficial ownership of such options.

Consists of 11,250 shares of shares of common stock underlying options that are exercisable as of April 7, 2016 or will become exercisable within 60 days after such date. Dr. Betz holds such options as a nominee of the CHOP.

- (5) will become exercisable within 60 days after such date. Dr. Betz holds such options as a nominee of the CHOP Foundation, which shall receive any economic benefit associated with such options. Dr. Betz disclaims beneficial ownership of such options.
- (6) Consists of 16,276 shares of shares of common stock underlying options that are exercisable as of April 7, 2016 or will become exercisable within 60 days after such date.
 - Consists of the shares of common stock and options described in note 11 below. Dr. Mehra is a managing member of Sofinnova Management VIII, L.L.C., the sole general partner of Sofinnova Venture Partners VIII, L.P.
- (7) Dr. Mehra may be deemed to have voting and investment power over the shares held by Sofinnova Venture Partners VIII, L.P. Dr. Mehra disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein.
- (8) Consists of 16,276 shares of shares of common stock underlying options that are exercisable as of April 7, 2016 or will become exercisable within 60 days after such date.
 - Consists of (a) 11,214 shares of common stock owned by Dr. Sigal, (b) 31,559 shares of common stock owned by Sigal Family Investments, LLC and (c) 20,419 shares of common stock underlying options that are exercisable as
- (9) of April 7, 2016 or will become exercisable within 60 days after such date. Dr. Sigal is a manager of Sigal Family Investments, LLC. Dr. Sigal may be deemed to have voting and investment power over the shares held by Sigal Family Investments, LLC. Dr. Sigal disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein.
 - Consists of (a) 7,767,379 shares of common stock owned by the CHOP Foundation, (b) 16,276 shares of common stock underlying options that are exercisable as of April 7, 2016 or will become exercisable within 60 days after such date issued to Dr. Altschuler as a nominee of the CHOP Foundation and (c) 11,250 shares of shares of common stock underlying options that are exercisable as of April 7, 2016 or will become exercisable within 60
- (10) days after such date issued to Dr. Betz as a nominee of the CHOP Foundation. The CHOP Foundation's board of trustees, or a committee designated by the board of trustees, has voting and investment power of their shares. The address of the CHOP Foundation is 34th Civic Center Boulevard, Philadelphia, PA 19104. We obtained much of the information regarding beneficial ownership of these shares from a Schedule 13D that was filed with the SEC on February 5, 2015.
 - All shares are held by FMR, LLC. Each of Fidelity Management & Research Company, Pyramis Global Advisors Trust Company, Edward C. Johnson 3rd, and members of the family of Edward C. Johnson 3rd may be deemed to
- (11) have shared voting power and shared dispositive power with respect to all shares held by FMR, LLC. The address for FMR, LLC is 245 Summer Street, Boston, Massachusetts 02210. Beneficial ownership is derived from a Schedule 13G/A filed on February 12, 2016.
 - Based on information provided in a Schedule 13G filed on February 12, 2016. T. Rowe Price Associates, Inc. ("Price Associates") does not serve as custodian of the assets of any of its clients; accordingly, in each instance only the client or the client's custodian or trustee bank has the right to receive dividends paid with respect to, and
- (12) proceeds from the sale of, such securities. The ultimate power to direct the receipt of dividends paid with respect to, and the proceeds from the sale of, such securities, is vested in the individual and institutional clients which Price Associates serves as investment adviser. Any and all discretionary authority which has been delegated to Price Associates may be revoked in whole or in part at any time.
- (13) Consists of (a) 1,566,071 shares of common stock owned by Sofinnova Venture Partners VIII, L.P. ("SVP VIII") and (b) 16,276 shares of common stock underlying options that are exercisable as of April 7, 2016 or will become exercisable within 60 days after such date owned by Dr. Mehra. Sofinnova Management VIII, L.L.C. ("SM VIII") is the general partner of SVP VIII and Anand Mehra, Michael Powell, Srinivas Akkarju and James I. Healy, are the managing members of SM VIII (the "Managing Members"). SVP VIII, SM VIII and the Managing Members may be deemed to have shared voting and dispositive power over the shares owned by SVP VIII. Such persons and

entities disclaim beneficial ownership over the shares owned by SVP VIII except to the extent of any pecuniary interest therein. The address of each such person or entity is Sofinnova Ventures, 3000 Sand Hill Road, Bldg. 4, Suite 250, Menlo Park, CA 94025. We obtained much of the information regarding beneficial ownership of these shares from a Schedule 13D/A that was filed with the SEC on October 21, 2015.

CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS AND DIRECTOR INDEPENDENCE

During 2015, we have engaged in the following transactions with our directors, executive officers and holders of more than 5% of our voting securities and affiliates of our directors, executive officers and holders of more than 5% of our voting securities. We believe that all of the transactions described below were made on terms no less favorable to us than could have been obtained from unaffiliated third parties.

Ongoing collaboration with CHOP

In October 2013, we entered into a licensing agreement with CHOP, which we amended in December 2013, May 2014, December 2014 and October 2015. We also entered into a technology assignment agreement, a master research service agreement and an administrative services agreement with CHOP in October 2013, a sponsored research agreement with CHOP in October 2014 and multiple clinical trial agreements with CHOP at various times regarding the conduct of our various clinical trials. We also entered into an additional licensing agreement with CHOP in November 2015, which supplements our existing license agreement with CHOP by granting us a worldwide exclusive license, with the right to sublicense, to use and practice a provisional patent application related to the production of gene therapies. Pursuant to these agreements, we paid CHOP \$5.2 million for the year ended December 31, 2015. Steven Altschuler, M.D., chairman of our Board of Directors, previously served as the chief executive officer of CHOP and the CHOP Foundation.

Under CHOP's Patent and Intellectual Property Policy (the "CHOP Policy") inventors have a right to a portion of any payments received by CHOP with respect to which they are listed as an inventor on a patent or patent application. In November 2015, Dr. High, our President and Chief Scientific Officer, and former CHOP employee, received \$1,508,990 from CHOP as a payment, in accordance with the CHOP Policy, reflecting Dr. High's status as an inventor on the patents or patent applications that were licensed by Spark from CHOP.

Participation in initial public offering

In our initial public offering, certain of our directors, executive officers and 5% stockholders and their affiliates purchased an aggregate of 560,700 shares of our common stock. Each of those purchases was made through the underwriters at the initial public offering price of \$23.00 per share. The following table sets forth the aggregate number of shares of our common stock that these directors, executive officers and 5% stockholders and their affiliates purchased in our initial public offering:

Purchaser(1)	Shares of	Total
ruicilasei(1)	common stock	purchase price
CHOP Foundation(2)	100,000	\$ 2,300,000
Sofinnova Venture Partners VIII, L.P.(3)	225,000	\$ 5,175,000
Entities affiliated with Baker Bros. Advisors LP	225,000	\$ 5,175,000
Elliott Sigal, M.D., Ph.D.	5,000	\$ 115,000
Stephen W. Webster	2,500	\$ 57,500
Rogério Vivaldi, M.D.	2,500	\$ 57,500
Katherine A. High, M.D.(4)	700	\$ 16,100

- (1) See "Security Ownership of Certain Beneficial Owners, Management and Related Stockholder Matters" for more information about the shares held by these entities.
- (2) Dr. Steven Altschuler, chairman of our Board of Directors, previously served as the chief executive officer of CHOP and the CHOP Foundation, a holder of more than 5% of our voting securities.
 - Dr. Anand Mehra, a member of our Board of Directors, is a managing member of Sofinnova Management VIII, L.L.C., the sole general partner of Sofinnova Venture Partners VIII, L.P., a holder of more than 5% of our voting
- (3) securities. Dr. Mehra may be deemed to have voting and investment power over the shares held by Sofinnova Venture Partners VIII, L.P. Dr. Mehra disclaims beneficial ownership of such shares except to the extent of any respective pecuniary interest therein.
- (4) Shares were purchased by Dr. High's husband.

Participation in follow-on offering

In our follow-on offering in December 2015, we offered 2,266,995 shares of common stock and CHOP offered 1,131,505 shares of our common stock. Affiliates of FMR LLC and T. Rowe Price Associates, Inc., holders of more than 5% of our voting securities, purchased 300,000 and 705,350 shares, respectively, of our common stock in this offering through the underwriters a the offering price of \$47.00 per share. No other directors, executive officers or 5% stockholders or their affiliates purchased stock in this follow-on offering.

Investors' rights agreement

We are a party to an investors' rights agreement, dated May 23, 2014, which we refer to as the Investors' Rights Agreement, with certain of our stockholders who purchased shares of our Series A Preferred Stock and Series B Preferred Stock, which includes our 5% stockholders and entities affiliated with two of our directors. The Investors' Rights Agreement provides these holders the right to demand that we file a registration statement or request that their shares be covered by a registration statement that we are otherwise filing.

Indemnification arrangements

Our Restated Certificate of Incorporation provides that we will indemnify our directors and officers to the fullest extent permitted by Delaware law. In addition, we have entered into indemnification agreements with each of our directors and executive officers that may be broader in scope than the specific indemnification provisions contained in the DGCL. See the "Executive Compensation—Limitation of liability and indemnification" section of this proxy for a further discussion of these arrangements.

SECTION 16(a) BENEFICIAL OWNERSHIP REPORTING COMPLIANCE

Section 16(a) of the Exchange Act requires our directors, executive officers and persons who own more than 10% of the Company's common stock to file with the SEC reports of ownership and changes in ownership of our common stock. Such persons are required by regulations of the SEC to furnish the Company with copies of all such filings. Based on our review of the reports we have received, Spark believes that all Section 16(a) filing requirements were complied with during the fiscal year ended December 31, 2015, except that a Form 4 for Katherine High for transactions on December 1, 2015 was filed before the market opened on December 4, 2015.

AUDIT COMMITTEE REPORT (1)

The audit committee has reviewed the Company's audited financial statements for the fiscal year ended December 31, 2015 and has discussed these statements with management and representatives of KPMG LLP, the Company's independent registered public accounting firm. Company management is responsible for the preparation of the Company's financial statements and for maintaining an adequate system of disclosure controls and procedures and internal control over financial reporting for that purpose. KPMG LLP is responsible for expressing an opinion on the conformity of the audited financial statements with generally accepted accounting principles, their judgments as to the quality, not just the acceptability, of the Company's accounting principles and such other matters as are required to be discussed with the audit committee under generally accepted auditing standards. The audit committee is responsible for providing independent, objective oversight of the Company's accounting functions and internal controls. The audit committee also received from, and discussed with, members of KPMG LLP the written disclosures and other communications that the Company's independent registered public accounting firm is required to provide to the audit committee, including the matters required to be discussed by Auditing Standard No. 16 (AS 16) (Communication with Audit Committees), as adopted by the Public Company Accounting Oversight Board, or PCAOB AS No. 16 requires our independent registered public accounting firm to discuss with the audit committee, among other things, the following:

methods to account for significant unusual transactions;

the effect of significant accounting policies in controversial or emerging areas for which there is a lack of authoritative guidance or consensus;

the process used by management in formulating particularly sensitive accounting estimates and the basis for the independent registered public accounting firm's conclusions regarding the reasonableness of those estimates; and disagreements with management regarding financial accounting and reporting matters and audit procedures.

KPMG LLP also provided the audit committee with the written disclosures and the letter required by Rule 3526 of the PCAOB. PCAOB Rule 3526 requires independent registered public accounting firms annually to disclose in writing all relationships that in their professional opinion may reasonably be thought to bear on independence, to confirm their perceived independence and engage in a discussion of independence. The audit committee has reviewed this disclosure and has discussed with members of KPMG LLP their independence from the Company.

Based on its discussions with management and our independent registered public accounting firm, and its review of the representations and information provided by management and our independent registered public accounting firm, the audit committee recommended to the Board of Directors that the audited financial statements be included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2015, for filing with the Securities and Exchange Commission.

Respectfully submitted by the Audit Committee,

Lota Zoth, CPA, Chairperson Lars Ekman, M.D., Ph.D. Anand Mehra, M.D. Vincent Milano

This Section is not "soliciting material," is not deemed filed with the SEC and is not to be incorporated by reference (1) in any filing of the Company under the Securities Act of 1933 or the Securities Exchange Act of 1934, whether made before or after the date hereof and irrespective of any general incorporation language in any such filing.

PROPOSAL 2: RATIFICATION OF SELECTION OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

The firm of KPMG LLP, independent registered public accounting firm, has been selected by the audit committee as auditors for Spark for the fiscal year ending December 31, 2016. KPMG LLP acted as the independent registered

public accounting firm for the Company for the years ended December 31, 2014 and 2015. A representative of KPMG LLP is expected to be present at

the Annual Meeting with the opportunity to make a statement if he or she or they desires and to respond to appropriate questions.

The Company's organizational documents do not require that the stockholders ratify the selection of KPMG LLP as the Company's independent registered public accounting firm. Spark requests such ratification as a matter of good corporate practice. A majority of the votes properly cast is required for the approval of the ratification of the selection of KPMG LLP as our independent registered public accounting firm, and brokers, bankers and other nominees have discretionary voting power on this routine matter. Abstentions and broker non-votes will have no effect on the ratification. If the stockholders do not ratify the selection, the audit committee will reconsider whether to retain KMPG LLP, but still may retain this firm. Even if the selection is ratified, the audit committee, in its discretion, may change the appointment at any time during the year if it determines that such a change would be in the best interests of the Company and its stockholders.

The audit committee, or a designated member thereof, pre-approves each audit and non-audit service rendered by KPMG LLP to Spark.

AUDITOR'S FEES

The following table summarizes the fees of KPMG LLP, our independent registered public accounting firm, billed to us for each of the last two fiscal years.

Fee Category	2014	2015
Audit Fees (1)	\$980,000	410,000
Audit-related Fee	es—	_
Tax Fees (2)	24,235	56,370
All Other Fees		_

Total Fees \$1,004,235 466,370

- (1) Audit fees consist of fees for the audit of our financial statements, the review of our interim financial statements and services associated with our registration statements on Form S-1.
- (2) Tax fees consists of fees incurred for tax compliance, tax advice and tax planning and includes fees for tax return preparation and tax consulting.

The aggregate fees included in the Audit Fees are billed for the fiscal year. The aggregate fees included in the Audit-related fees and Tax Fees are fees billed in the fiscal year.

All such accountant services and fees were pre-approved by our audit committee in accordance with the "Pre-Approval Policies" described below.

Pre-approval policies

The audit committee of our Board of Directors has adopted policies and procedures for the pre-approval of audit and non-audit services for the purpose of maintaining the independence of our independent auditor. We may not engage our independent auditor to render any audit or non-audit service unless either the service is approved in advance by the audit committee, or the engagement to render the service is entered into pursuant to the audit committee's pre-approval policies and procedures.

THE BOARD OF DIRECTORS UNANIMOUSLY RECOMMENDS THAT YOU VOTE FOR

THE RATIFICATION OF THE SELECTION OF KPMG LLP AS THE COMPANY'S INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM FOR THE FISCAL YEAR ENDING DECEMBER 31, 2016 (PROPOSAL 2 ON YOUR PROXY CARD)

POLICIES ON REPORTING OF CONCERNS REGARDING ACCOUNTING AND OTHER MATTERS AND ON COMMUNICATING WITH NON-MANAGEMENT DIRECTORS

The Board of Directors and the audit committee have adopted policies on reporting concerns regarding accounting and other matters and on communicating with the non-management directors. Any person, whether or not an employee, who has a

concern about the conduct of Spark, or any of its people, including with respect to accounting, internal accounting controls or auditing matters, may, in a confidential or anonymous manner, communicate that concern to the Company's General Counsel, who is the designated contact for these purposes. Contact may be made:

By e-mail to ONCE@openboard.info (anonymity can be maintained);

In writing (anonymity cannot be maintained), addressed to the General Counsel by U.S. mail to c/o Spark Therapeutics, Inc., 3737 Market Street, Suite 1300, Philadelphia, PA 19104;

Online at http://www.openboard.info/ONCE/ (which may be done anonymously as set forth on the online instructions); or

By phoning a voicemail account that we have established for receipt of questions and reports of potential violations of the Code of conduct and ethics. The voicemail account may be reached at (866) 822-6487 and calls may be made anonymously as set forth in the online instructions at http://www.openboard.info/ONCE/. Any interested party, whether or not an employee, who wishes to communicate directly with the audit committee, also may contact the audit committee using one of the above methods.

Code of conduct and ethics

We have adopted a written code of business conduct and ethics that applies to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller or persons performing similar functions. We have posted on our website, www.sparktx.com, a current copy of the code and all disclosures that are required by law or NASDAQ stock market listing standards concerning any amendments to, or waivers from, any provision of the code.

WHERE YOU CAN FIND MORE INFORMATION

The Company files annual, quarterly and current reports, proxy statements and other information with the SEC. You may read and copy any reports, statements, or other information that Company files at the SEC's public reference room at the following location: 100 F Street, N.E., Washington, D.C. 20549.

Please call the SEC at 1-800-732-0330 for further information on the public reference room. The Company's SEC filings are also available to the public from commercial document retrieval services and at the website maintained by the SEC at www.sec.gov. You may also read and copy any document the Company files with the SEC on our website at www.sparktx.com under the "Investors" menu.

You should rely on the information contained in this document to vote your shares at the Annual Meeting. Spark has not authorized anyone to provide you with information that is different from what is contained in this document. This document is dated April 21, 2016. You should not assume that the information contained in this document is accurate as of any date other than that date, and the mailing of this document to stockholders at any time after that date does not create an implication to the contrary. This Proxy Statement does not constitute a solicitation of a proxy in any jurisdiction where, or to or from any person to whom, it is unlawful to make such proxy solicitations in such jurisdiction.

FORM 10-K

We are subject to the informational requirements of the Exchange Act and, in accordance therewith, file reports, proxy statements and other information with the SEC. Reports, proxy statements and other information filed by us may be inspected without charge and copies obtained upon payment of prescribed fees from the Public Reference Section of the SEC at 100 F Street, NE, Washington, D.C. 20549, or by way of the SEC's website, www.sec.gov.

We will provide without charge to each person to whom a copy of the proxy statement is delivered, upon the written or oral request of any such persons, additional copies of our Annual Report on Form 10-K, for the fiscal year ended December 31, 2015 as filed with the SEC. Requests for such copies should be addressed to:

Spark Therapeutics, Inc.

3737 Market Street

Suite 1300

Philadelphia, PA 19104

(855) 772-7589

Attention: Joseph W. La Barge, Secretary

HOUSEHOLDING OF ANNUAL MEETING MATERIALS

Stockholders of Spark common stock who share a single address, may receive only one copy of this Proxy Statement, Notice of Internet Availability and our Annual Report on Form 10-K for the fiscal year ended December 31, 2015, unless Spark has received contrary instructions from any stockholder at that address. This practice, known as "householding," is designed to reduce the Company's printing and postage costs. However, if any stockholder residing at such an address wishes to receive a separate copy of this Proxy Statement, Notice of Internet Availability or our Annual Report on Form 10-K for the fiscal year ended December 31, 2015, he or she may contact Spark Therapeutics, Inc., 3737 Market Street, Suite 1300, Philadelphia, PA 19104, (855) 772-7589, Attention: Joseph W. La Barge, Secretary, and Spark will deliver those documents to such stockholder promptly upon receiving the request. Any such stockholder may also contact Joseph W. La Barge, Secretary, using the above contact information if he or she would like to receive separate proxy statements, notice of internet availability and annual reports in the future. If you are receiving multiple copies of our annual reports, notice of internet availability and proxy statements, you may request householding in the future by contacting Joseph W. La Barge, Secretary.

OTHER BUSINESS

The Board of Directors knows of no business to be brought before the Annual Meeting which is not referred to in the accompanying Notice of Annual Meeting. Should any such matters be presented, the persons named in the proxy shall have the authority to take such action in regard to such matters as in their judgment seems advisable. If you hold shares through a broker, bank or other nominee as described above, they will not be able to vote your shares on any other business that comes before the Annual Meeting unless they receive instructions from you with respect to such matter.