

Ultragenyx Pharmaceutical Inc.  
Form S-1/A  
December 23, 2013

As filed with the Securities and Exchange Commission on December 23, 2013.

Registration No. 333-192244

**UNITED STATES**  
**SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

**Amendment No. 1**

**to**

**FORM S-1**

**REGISTRATION STATEMENT UNDER THE SECURITIES ACT OF 1933**

**ULTRAGENYX PHARMACEUTICAL INC.**

*(Exact name of Registrant as specified in its charter)*

**Delaware**

*(State or other jurisdiction of  
incorporation or organization)*

**2834**

*(Primary Standard Industrial  
Classification Code Number)*  
**60 Leveroni Court**

**27-2546083**

*(I.R.S. Employer  
Identification Number)*

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Novato, CA 94949

(415) 483-8800

*(Address, including zip code, and telephone number, including area code, of Registrant's principal executive offices)*

**Emil D. Kakkis, M.D., Ph.D.**

**President and Chief Executive Officer**

**Ultragenyx Pharmaceutical Inc.**

**60 Leveroni Court**

**Novato, CA 94949**

**(415) 483-8800**

*(Name, address, including zip code, and telephone number, including area code, of agent for service)*

**Copies to:**

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Chief Financial Officer  
Ultragenyx Pharmaceutical Inc.  
60 Leveroni Court  
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**B. Shayne Kennedy, Esq.  
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**Approximate date of commencement of proposed sale to the public:**

As soon as practicable after the effective date of this Registration Statement.

If any of the securities being registered on this Form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933, check the following box. "

If this Form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, please check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. "

If this Form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. "

If this Form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. "

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

Large accelerated filer       Accelerated filer       Non-accelerated filer       Smaller reporting company   
(Do not check if a smaller reporting company)

**The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933 or until the Registration Statement shall become effective on such date as the Commission, acting pursuant to said Section 8(a), may determine.**

**Explanatory Note**

This Amendment No. 1 is being filed for the purpose of filing Exhibits 10.1, 10.2, 10.3, 10.6, 10.7, 10.8 and 10.9. No changes or additions are being made hereby to the Prospectus constituting Part I of the Registration Statement (not included herein) or to Part II of the Registration Statement other than with respect to Item 15 of Part II.

**PART II****Information Not Required in Prospectus****Item 13. Other Expenses of Issuance and Distribution.**

The following table sets forth the costs and expenses, other than the underwriting discounts and commissions, payable by the registrant in connection with the sale of common stock being registered. All amounts are estimates except for the Securities and Exchange Commission, or SEC, registration fee, the FINRA filing fee and The NASDAQ Global Market listing fee.

| Item                                      | Amount<br>to be paid |
|---|----------------------|
| SEC registration fee                      | \$ 11,109            |
| FINRA filing fee                          | 13,438               |
| The NASDAQ Global Market Listing fee      | *                    |
| Printing and engraving expenses           | *                    |
| Legal fees and expenses                   | *                    |
| Accounting fees and expenses              | *                    |
| Blue Sky, qualification fees and expenses | *                    |
| Transfer Agent fees and expenses          | *                    |
| Miscellaneous expenses                    | *                    |
| <br>Total                                 | <br>\$ *             |

\* To be provided by amendment

**Item 14. Indemnification of Directors and Officers.**

Section 145(a) of the Delaware General Corporation Law provides, in general, that a corporation may indemnify any person who was or is a party or is threatened to be made a party to any threatened, pending or completed action, suit or proceeding, whether civil, criminal, administrative or investigative (other than an action by or in the right of the corporation), because he or she is or was a director, officer, employee or agent of the corporation, or is or was serving at the request of the corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise, against expenses (including attorneys' fees), judgments, fines and amounts paid in settlement actually and reasonably incurred by the person in connection with such action, suit or proceeding, if he or she acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of the corporation and, with respect to any criminal action or proceeding, had no reasonable cause to believe his or her conduct was unlawful.

Section 145(b) of the Delaware General Corporation Law provides, in general, that a corporation may indemnify any person who was or is a party or is threatened to be made a party to any threatened, pending or completed action or suit by or in the right of the corporation to procure a judgment in its favor because the person is or was a director, officer, employee or agent of the corporation, or is or was serving at the request of the corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise, against expenses (including attorneys' fees) actually and reasonably incurred by the person in connection with the defense or settlement of such action or suit if he or she acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of the corporation, except that no indemnification shall be made with respect to any claim, issue or matter as to which he or she shall have been adjudged to be liable to the corporation unless and only to the extent that the Court of Chancery or other adjudicating court determines that, despite the adjudication of liability but in view of all of the circumstances of the case, he or she is fairly and reasonably entitled to indemnity for such expenses which the Court of Chancery or other adjudicating court shall deem proper.

Section 145(g) of the Delaware General Corporation Law provides, in general, that a corporation may purchase and maintain insurance on behalf of any person who is or was a director, officer, employee or agent of



the corporation, or is or was serving at the request of the corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise against any liability asserted against such person and incurred by such person in any such capacity, or arising out of his or her status as such, whether or not the corporation would have the power to indemnify the person against such liability under Section 145 of the Delaware General Corporation Law.

Article VI of our amended and restated certificate of incorporation (the Charter), provides that no director of our company shall be personally liable to us or our stockholders for monetary damages for any breach of fiduciary duty as a director, except for liability (1) for any breach of the director's duty of loyalty to us or our stockholders, (2) for acts or omissions not in good faith or which involve intentional misconduct or a knowing violation of law, (3) in respect of unlawful dividend payments or stock redemptions or repurchases, or (4) for any transaction from which the director derived an improper personal benefit. In addition, our Charter provides that if the Delaware General Corporation Law is amended to authorize the further elimination or limitation of the liability of directors, then the liability of a director of our company shall be eliminated or limited to the fullest extent permitted by the Delaware General Corporation Law, as so amended.

Article VI of the Charter further provides that any repeal or modification of such article by our stockholders or amendment to the Delaware General Corporation Law will not adversely affect any right or protection existing at the time of such repeal or modification with respect to any acts or omissions occurring before such repeal or modification of a director serving at the time of such repeal or modification.

In connection with the sale of common stock being registered hereby, we have entered into indemnification agreements with each of our directors and our executive officers. These agreements will provide that we will indemnify each of our directors and such officers to the fullest extent permitted by law and the Charter.

We also maintain an insurance policy that covers certain liabilities of directors and officers of our company arising out of claims based on acts or omissions in their capacities as directors or officers.

In any underwriting agreement we enter into in connection with the sale of common stock being registered hereby, attached hereto as exhibit 1.1, the underwriters will agree to indemnify, under certain conditions, us, our directors, our officers and persons who control us within the meaning of the Securities Act against certain liabilities.

**Item 15. Recent Sales of Unregistered Securities.**

In the three years preceding the filing of this registration statement, we or our California corporation predecessor have issued the following securities that were not registered under the Securities Act. Issuances made prior to June 2011 were made by Ultragenyx Pharmaceutical, Inc., a California corporation. In connection with our reincorporation into Delaware in June 2011, each outstanding share of common stock was converted into 80 shares of the new Delaware corporation.

*Common Stock Purchase Agreements*

On April 27, 2010, we issued 100,000 shares of common stock for consideration of \$0.01 per share, for an aggregate purchase price of \$1,000.00 to Emil D. Kakkis. Emil D. Kakkis is our current President and Chief Executive Officer and one of our directors.

On November 19, 2010, we issued 3,000 shares of common stock for consideration of \$0.01 per share, for an aggregate purchase price of \$30.00 to Nobelpharma Co. Ltd.

On February 11, 2011, we issued 1,030 shares of common stock for consideration of \$0.01 per share, for an aggregate purchase price of \$10.30 to William E. Aliski. Mr. Aliski is one of our directors.

On February 28, 2011, we issued 1,030 shares of common stock for consideration of \$0.01 per share, for an aggregate purchase price of \$10.30 to Jonathan K. Wright.

On March 11, 2011, we issued 1,667 shares of common stock for consideration of \$0.01 per share, for an aggregate purchase price of \$16.67 to William E. Aliski. Mr. Aliski is one of our directors.

On March 28, 2011, we issued 1,030 shares of common stock for consideration of \$0.10 per share, for an aggregate purchase price of \$103.00 to Steven Jungles. Mr. Jungles is our Senior Vice President, Technical Operations.

On April 6, 2011, we issued 11,300 shares of common stock for consideration of \$0.01 per share, for an aggregate purchase price of \$113.00 to John Klock.

We claimed exemption from registration under the Securities Act for the sale and issuance of these shares of common stock by virtue of Section 4(a)(2) and/or Regulation D promulgated thereunder as transactions not involving any public offering. All of the purchasers of the shares of common stock for which we relied on Section 4(a)(2) and/or Regulation D represented that they were accredited investors as defined under the Securities Act. We claimed such exemption on the basis that (a) the purchasers in each case represented that they intended to acquire the securities for investment only and not with a view to the distribution thereof and that they either received adequate information about the registrant or had access, through employment or other relationships, to such information and (b) appropriate legends were affixed to the stock certificates issued in such transactions.

#### *Convertible Notes and Series A Convertible Preferred Stock Warrants*

On June 30, 2010, we entered into a Note and Warrant Purchase Agreement with Emil D. Kakkis, our President and Chief Executive Officer and one of our directors. Pursuant to the Note and Warrant Purchase Agreement, we issued a convertible promissory note in the amount of \$1.0 million to Dr. Kakkis and also issued him a warrant to purchase up to 241,803 shares of our Series A convertible preferred stock.

On February 15, 2011, we entered into a Note and Warrant Purchase Agreement with the John and Cynthia Klock Trust. Pursuant to the Note and Warrant Purchase Agreement, we issued a convertible promissory note in the amount of \$1.5 million to the John and Cynthia Klock Trust and also issued to the trust a warrant to purchase up to 507,786 shares of our Series A convertible preferred stock.

On February 23, 2011, we entered into a Note and Warrant Purchase Agreement with William Aliski, one of our directors. Pursuant to the Note and Warrant Purchase Agreement, we issued a convertible promissory note in the amount of \$250,000 to Mr. Aliski and also issued him a warrant to purchase up to 84,631 shares of our Series A convertible preferred stock.

On June 14, 2011, we entered into a Note and Warrant Purchase Agreement with Emil D. Kakkis, our President and Chief Executive Officer and one of our directors. Pursuant to the Note and Warrant Purchase Agreement, we issued a convertible promissory note in the amount of \$300,000 to Dr. Kakkis and also issued him a warrant to purchase up to 72,541 shares of our Series A convertible preferred stock.

On June 14, 2011, we entered into a second Note and Warrant Purchase Agreement with Emil D. Kakkis, our President and Chief Executive Officer and one of our directors. Pursuant to the Note and Warrant Purchase Agreement, we issued a convertible promissory note in the amount of \$500,000 to Dr. Kakkis and also issued him a warrant to purchase up to 120,901 shares of our Series A convertible preferred stock.

We claimed exemption from registration under the Securities Act for the sale and issuance of these securities by virtue of Section 4(a)(2) and/or Regulation D promulgated thereunder as transactions not involving any public offering. All of the purchasers of unregistered securities for which we relied on Section 4(a)(2) and/or Regulation D represented that they were accredited investors as defined under the Securities Act. We claimed such exemption on the basis that (a) the purchasers in each case represented that they intended to acquire the securities for investment only and not with a view to the distribution thereof and that they either received adequate information about the registrant or had access, through employment or other relationships, to such information and (b) appropriate legends were affixed to the stock certificates issued in such transactions.



*Series A Convertible Preferred Stock Financing*

On June 16, 2011, we sold an aggregate of 18,052,464 shares of our Series A convertible preferred stock to eight investors at a purchase price of \$1.034 per share, for an aggregate purchase price of approximately \$15.0 million in cash and \$3.7 million in converted bridge notes. On July 16, 2012, we sold, pursuant to a second tranche closing, an aggregate of 14,604,895 shares of our Series A convertible preferred stock to six investors at a purchase price of \$1.034 per share, for an aggregate purchase price of \$15.1 million in cash. We claimed exemption from registration under the Securities Act for the sale and issuance of these securities by virtue of Section 4(a)(2) and/or Regulation D promulgated thereunder as transactions not involving any public offering. All of the purchasers of unregistered securities for which we relied on Section 4(a)(2) and/or Regulation D represented that they were accredited investors as defined under the Securities Act. We claimed such exemption on the basis that (a) the purchasers in each case represented that they intended to acquire the securities for investment only and not with a view to the distribution thereof and that they either received adequate information about the registrant or had access, through employment or other relationships, to such information and (b) appropriate legends were affixed to the stock certificates issued in such transactions.

*Series A Paid-in-Kind Dividends*

On August 16, 2012, we issued an aggregate of 1,193,088 shares of our Series A convertible preferred stock to holders of our Series A convertible preferred stock in accordance with Article IV.B.1.(a) of our Certificate of Incorporation that was filed with the Secretary of State of the State of Delaware on June 13, 2011. We claimed exemption from registration under the Securities Act for the sale and issuance of these securities by virtue of Section 4(a)(2) and/or Regulation D promulgated thereunder as transactions not involving any public offering.

On December 14, 2012, we issued an aggregate of 499,447 shares of our Series A convertible preferred stock to holders of our Series A convertible preferred stock in accordance with Article IV.B.1.(a) of our Certificate of Incorporation that was filed with the Secretary of State of the State of Delaware on June 13, 2011. We claimed exemption from registration under the Securities Act for the sale and issuance of these securities by virtue of Section 4(a)(2) and/or Regulation D promulgated thereunder as transactions not involving any public offering.

*Series B Convertible Preferred Stock Financing*

On December 18, 2012, we sold an aggregate of 27,081,680 shares of our Series B convertible preferred stock to 34 investors at a purchase price of \$2.7694 per share, for an aggregate purchase price of approximately \$75 million in cash. We claimed exemption from registration under the Securities Act for the sale and issuance of these securities by virtue of Section 4(a)(2) and/or Regulation D promulgated thereunder as transactions not involving any public offering. All of the purchasers of unregistered securities for which we relied on Section 4(a)(2) and/or Regulation D represented that they were accredited investors as defined under the Securities Act. We claimed such exemption on the basis that (a) the purchasers in each case represented that they intended to acquire the securities for investment only and not with a view to the distribution thereof and that they either received adequate information about the registrant or had access, through employment or other relationships, to such information and (b) appropriate legends were affixed to the stock certificates issued in such transactions.

*Stock Options*

From November 17, 2011 through December 19, 2013, we granted stock options to employees under our 2011 Equity Incentive Plan, as amended, covering an aggregate of 10,089,000 shares of common stock, at a weighted-average average exercise price of \$0.81 per share. Of these, options covering an aggregate of 821,667 shares were cancelled without being exercised and we sold an aggregate of 2,280,945 shares of common stock to employees for cash consideration in the aggregate amount of \$0.3 million upon the exercise of stock options.

We claimed exemption from registration under the Securities Act for these sales and issuances under Section 4(a)(2) of the Securities Act in that such sales and issuances did not involve a public offering or under

Rule 701 promulgated under the Securities Act, in that they were offered and sold either pursuant to written compensatory plans or pursuant to a written contract relating to compensation, as provided by Rule 701.

**Item 16. Exhibits and Financial Statement Schedules.**

**(a) Exhibits.**

See the Exhibit Index attached to this Registration Statement, which is incorporated by reference herein.

**(b) Financial Statement Schedules.**

Schedules not listed above have been omitted because the information required to be set forth therein is not applicable or is shown in the financial statements or notes thereto.

**Item 17. Undertakings.**

The undersigned Registrant hereby undertakes to provide to the underwriters at the closing specified in the underwriting agreement certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers and controlling persons of the Registrant pursuant to the foregoing provisions, or otherwise, the Registrant has been advised that in the opinion of the SEC such indemnification is against public policy as expressed in the Securities Act, and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the Registrant of expenses incurred or paid by a director, officer, or controlling person of the Registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the Registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question of whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.

The undersigned Registrant hereby undertakes that:

1. For purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this Registration Statement in reliance upon Rule 430A and contained in a form of prospectus filed by the Registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this Registration Statement as of the time it was declared effective.
2. For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

The undersigned Registrant hereby undertakes to provide to the underwriters at the closing specified in the underwriting agreement certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

**Signatures**

Pursuant to the requirements of the Securities Act of 1933, as amended, the Registrant has duly caused this Amendment No. 1 to Registration Statement on Form S-1 to be signed on its behalf by the undersigned, thereunto duly authorized, in Novato, California, on December 23, 2013.

**ULTRAGENYX PHARMACEUTICAL INC.**

By: /s/ EMIL D. KAKKIS  
 Emil D. Kakkis, M.D., Ph.D.  
 President and Chief Executive Officer

Pursuant to the requirements of the Securities Act, this Amendment No. 1 to Registration Statement has been signed by the following persons in the capacities and on the dates indicated.

| <b>Signature</b>                                  | <b>Title</b>  | <b>Date</b>       |
|---|---|-------------------|
| /s/ EMIL D. KAKKIS<br>Emil D. Kakkis, M.D., Ph.D. | Director, President and Chief Executive Officer<br><i>(Principal Executive Officer)</i>               | December 23, 2013 |
| /s/ SHALINI SHARP<br>Shalini Sharp                | Senior Vice President, Chief Financial Officer<br><i>(Principal Financial and Accounting Officer)</i> | December 23, 2013 |
| *   | Chairman of the Board   | December 23, 2013 |
| Eran Nadav, Ph.D.<br>*                            | Director  | December 23, 2013 |
| Benjamin Auspitz<br>*                             | Director  | December 23, 2013 |
| Mårten Steen, M.D., Ph.D.<br>*                    | Director  | December 23, 2013 |
| William Aliski                                    |   |                   |

\*By: /s/ SHALINI SHARP  
 Shalini Sharp  
 Attorney-in-fact

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**Exhibit Index**

| Exhibit Number | Exhibit Description  | Incorporated by Reference |           |        | Filed Herewith |
|----------------|--|---------------------------|-----------|--------|----------------|
|                |  | Form                      | Date      | Number |                |
| 1.1*           | Form of Underwriting Agreement   |                           |           |        |                |
| 3.1            | Amended and Restated Certificate of Incorporation, as currently in effect  | S-1                       | 11/8/2013 | 3.1    |                |
| 3.2            | Bylaws, as currently in effect   | S-1                       | 11/8/2013 | 3.2    |                |
| 3.3*           | Amended and Restated Certificate of Incorporation, to be in effect immediately prior to the completion of this offering                              |                           |           |        |                |
| 3.4*           | Amended and Restated Bylaws, to be in effect immediately prior to the completion of this offering  |                           |           |        |                |
| 4.1            | Reference is made to Exhibits 3.1 through 3.4  |                           |           |        |                |
| 4.2            | Form of Common Stock Certificate   | S-1                       | 11/8/2013 | 4.2    |                |
| 4.3            | Warrant to purchase Series A Preferred Stock of the Registrant, dated as of June 30, 2010, issued to Emil D. Kakkis, M.D., Ph.D.                     | S-1                       | 11/8/2013 | 4.3    |                |
| 4.4            | Warrant to purchase Series A Preferred Stock of the Registrant, dated as of February 15, 2011, issued to the John and Cynthia Klock Charitable Trust | S-1                       | 11/8/2013 | 4.4    |                |
| 4.5            | Warrant to purchase Series A Preferred Stock of the Registrant, dated as of February 23, 2011, issued to William E. Aliski                           | S-1                       | 11/8/2013 | 4.5    |                |
| 4.6            | Warrant to purchase Series A Preferred Stock of the Registrant, dated as of June 14, 2011, issued to Emil D. Kakkis, M.D., Ph.D.                     | S-1                       | 11/8/2013 | 4.6    |                |
| 4.7            | Warrant to purchase Series A Preferred Stock of the Registrant, dated as of June 14, 2011, issued to Emil D. Kakkis, M.D., Ph.D.                     | S-1                       | 11/8/2013 | 4.7    |                |
| 4.8            | Amended and Restated Investors Rights Agreement, dated December 18, 2012, among the Registrant and the investors named therein                       | S-1                       | 11/8/2013 | 4.8    |                |
| 5.1*           | Opinion of Ropes & Gray LLP  |                           |           |        |                |
| 10.1           | Collaboration and License Agreement, dated as of August 29, 2013, between the Registrant and Kyowa Hakko Kirin Co., Ltd.                             |                           |           |        | X              |
| 10.2           | License Agreement, dated as of March 1, 2011, between the Registrant and AAIPharma Services Corp.  |                           |           |        | X              |
| 10.3           | License Agreement, dated as of September 20, 2012, between the Registrant and Baylor Research Institute  |                           |           |        | X              |
| 10.4           | Amendment to the License Agreement, dated as of March 22, 2013, between the Registrant and Baylor Research Institute                                 | S-1                       | 11/8/2013 | 10.4   |                |
| 10.5           | Exclusive License Agreement, dated as of April 23, 2012, between the Registrant and HIBM Research Group  | S-1                       | 11/8/2013 | 10.5   |                |
| 10.6           | Collaboration and License Agreement, dated as of September 30, 2010, between the Registrant and Nobelpharma Co., Ltd.                                |                           |           |        | X              |
| 10.7           | License Agreement, dated as of September 1, 2012, between the Registrant and St. Jude Children's Research Hospital                                   |                           |           |        | X              |

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| Exhibit Number | Exhibit Description   | Incorporated by Reference |           |        | Filed Herewith |
|----------------|---|---------------------------|-----------|--------|----------------|
|                |   | Form                      | Date      | Number |                |
| 10.8           | Exclusive License Agreement, dated as of November 22, 2010, between the Registrant and Saint Louis University   |                           |           |        | X              |
| 10.9           | Supply Agreement, dated as of November 19, 2012, between the Registrant and CREMER OLEO GmbH & Co KG  |                           |           |        | X              |
| 10.10          | Development and Clinical Supply Agreement, dated as of August 31, 2012, between the Registrant and Rentschler Biotechnologie GmbH                     | S-1                       | 11/8/2013 | 10.10  |                |
| 10.11#         | 2011 Equity Incentive Plan (including forms of Stock Option Grant Notice and Stock Option Agreement thereunder)                                       | S-1                       | 11/8/2013 | 10.11  |                |
| 10.12#         | Amendment to the 2011 Equity Incentive Plan   | S-1                       | 11/8/2013 | 10.12  |                |
| 10.13#*        | 2014 Incentive Plan   |                           |           |        |                |
| 10.14#*        | Form of Incentive Stock Option Award Agreement  |                           |           |        |                |
| 10.15#*        | Form of Non-Qualified Stock Option Award Agreement  |                           |           |        |                |
| 10.16#*        | Form of Restricted Stock Unit Award Agreement   |                           |           |        |                |
| 10.17#*        | 2014 Employee Stock Purchase Plan   |                           |           |        |                |
| 10.18#         | Executive Employment Agreement, dated as of June 15, 2011, between the Registrant and Emil D. Kakkis, M.D., Ph.D.                                     | S-1                       | 11/8/2013 | 10.18  |                |
| 10.19#         | Offer Letter, dated as of October 31, 2011, between the Registrant and Thomas Kassberg  | S-1                       | 11/8/2013 | 10.19  |                |
| 10.20#         | Offer Letter, dated March 12, 2012, between the Registrant and Shalini Sharp  | S-1                       | 11/8/2013 | 10.20  |                |
| 10.21#*        | Form of Indemnification Agreement   |                           |           |        |                |
| 10.22          | Standard Lease, dated as of July 5, 2011, between the Registrant and Condiotti Enterprises, Inc.  | S-1                       | 11/8/2013 | 10.22  |                |
| 10.23          | License and Services Agreement, dated as of September 24, 2010, between the Registrant and The Buck Institute for Age Research                        | S-1                       | 11/8/2013 | 10.23  |                |
| 10.24          | Amendment No. 1 to License and Services Agreement, dated as of September 4, 2012, between the Registrant and The Buck Institute for Research on Aging | S-1                       | 11/8/2013 | 10.24  |                |
| 10.25#*        | Corporate Bonus Plan  |                           |           |        |                |
| 23.1           | Consent of independent registered public accounting firm  | S-1                       | 11/8/2013 | 23.1   |                |
| 23.2*          | Consent of Ropes & Gray LLP (included in Exhibit 5.1)   |                           |           |        |                |
| 24.1           | Power of Attorney   | S-1                       | 11/8/2013 | 24.1   |                |
| 99.1           | Consent of Matthew Fust   | S-1                       | 11/8/2013 | 99.1   |                |

\* To be filed by amendment.

Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment and this exhibit has been filed separately with the SEC.

# Indicates management contract or compensatory plan.