Ultragenyx Pharmaceutical Inc.
Form 10-Q
August 03, 2018

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934.
For the quarterly period ended June 30, 2018

OR

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934.
For the transition period from to .

Commission File No. 001-36276

ULTRAGENYX PHARMACEUTICAL INC.

(Exact name of registrant as specified in its charter)

Delaware	27-2546083
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)

60 Leveroni Court	
Novato, California	94949
(Address of principal executive offices)	(Zip Code)

(415) 483-8800

(Registrant’s telephone number, including area code)

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days.
YES NO

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Website, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). YES NO

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of “large accelerated filer,” “accelerated filer,” “smaller reporting company,” and “emerging growth company” in Rule 12b-2 of the Exchange Act. (Check one):

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

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act).
YES NO

As of July 30, 2018, the registrant had 50,182,357 shares of common stock issued and outstanding.

ULTRAGENYX PHARMACEUTICAL INC.

FORM 10-Q FOR THE QUARTER ENDED JUNE 30, 2018

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CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q (the "Quarterly Report") contains forward-looking statements that involve risks and uncertainties. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this Quarterly Report are forward-looking statements. In some cases, you can identify forward-looking statements by words such as "anticipate," "believe," "contemplate," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "seek," "should," "target," "will," "would," or the negative of these w comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- our commercialization, marketing, and manufacturing capabilities and strategy;
- our expectations regarding the timing of clinical study commencements and reporting results from same;
- the timing and likelihood of regulatory approvals for our product candidates;
- the anticipated indications for our product candidates, if approved;
- the potential market opportunities for commercializing our products and product candidates;
- our expectations regarding the potential market size and the size of the patient populations for our products and product candidates, if approved for commercial use;
- estimates of our expenses, revenue, capital requirements, and our needs for additional financing;
- our ability to develop, acquire, and advance product candidates into, and successfully complete, clinical studies;
- the implementation of our business model and strategic plans for our business, products and product candidates and the integration and performance of any businesses we have acquired or may acquire;
    - the initiation, timing, progress, and results of ongoing and future preclinical and clinical studies, and our research and development programs;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our products and product candidates;
- our ability to maintain and establish collaborations or strategic relationships or obtain additional funding;
- our ability to maintain and establish relationships with third parties, such as contract research organizations, contract manufacturing organizations, suppliers, and distributors;
- our financial performance and the expansion of our organization;
- our ability to obtain supply of our products and product candidates;
- the scalability and commercial viability of our manufacturing methods and processes;
- developments and projections relating to our competitors and our industry; and
    - other risks and uncertainties, including those listed under Part II, Item 1A. Risk Factors.

Any forward-looking statements in this Quarterly Report reflect our current views with respect to future events or to our future financial performance and involve known and unknown risks, uncertainties, and other factors that may cause our actual results, performance, or achievements to be materially different from any future results, performance, or achievements expressed or implied by these forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those discussed under Part II, Item 1A. Risk Factors and discussed elsewhere in this Quarterly Report. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

This Quarterly Report also contains estimates, projections, and other information concerning our industry, our business, and the markets for certain diseases, including data regarding the estimated size of those markets, and the incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research, or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, we obtained this industry, business, market, and other data from reports, research surveys, studies, and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data, and similar sources.

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PART I. FINANCIAL INFORMATION

Item 1. Financial Statements

ULTRAGENYX PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

(Unaudited)

(In thousands, except share amounts)

	June 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$110,854	$ 100,488
Short-term investments	436,278	134,005
Accounts receivable	17,425	5,172
Inventory	3,342	757
Prepaid expenses and other current assets	36,844	29,161
Total current assets	604,743	269,583
Property and equipment, net	20,858	21,837
Long-term investments	—	9,975
Intangible assets, net	132,776	141,545
Goodwill	44,406	44,406
Other assets	3,239	3,407
Total assets	$806,022	$ 490,753
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$10,314	$ 8,886
Accrued liabilities	55,735	62,128
Total current liabilities	66,049	71,014
Deferred tax liabilities	31,166	31,166
Other liabilities	4,836	5,119
Total liabilities	102,051	107,299
Stockholders' equity:		
Preferred stock — 25,000,000 shares authorized; nil outstanding as of June 30, 2018 and December 31, 2017	—	—
Common stock — 250,000,000 shares authorized; 50,116,056 and 44,167,071 shares issued and outstanding as of June 30, 2018 and December 31, 2017, respectively	50	44
Additional paid-in capital	1,559,722	1,221,762
Accumulated other comprehensive loss	(654)	(5,680)
Accumulated deficit	(855,147)	(832,672)
Total stockholders' equity	703,971	383,454

Total liabilities and stockholders' equity	$806,022	$490,753

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

(Unaudited)

(In thousands, except share and per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Revenues:				
Collaboration and license	$10,519	$—	$19,881	$—
Product sales	2,275	—	3,590	—
Total revenues	12,794	—	23,471	—
Operating expenses:				
Cost of sales	141	—	366	—
Research and development	76,835	58,436	152,339	109,705
Selling, general and administrative	30,718	20,005	62,153	38,690
Total operating expenses	107,694	78,441	214,858	148,395
Loss from operations	(94,900)	(78,441)	(191,387)	(148,395)
Other income (expense), net:				
Interest income	2,448	1,151	4,185	2,233
Gain from sale of priority review vouchers	40,322	—	170,322	—
Other income (expense)	(496)	4,413	(5,454)	4,995
Loss before income taxes	(52,626)	(72,877)	(22,334)	(141,167)
Provision for income taxes	(102)	(14)	(141)	(14)
Net loss	$(52,728)	$(72,891)	$(22,475)	$(141,181)
Net loss per share, basic and diluted	$(1.06)	$(1.72)	$(0.46)	$(3.35)
Shares used in computing net loss per share, basic and diluted	49,819,528	42,346,830	49,046,838	42,095,750

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

(Unaudited)

(In thousands)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Net loss	$(52,728)	$(72,891)	$(22,475)	$(141,181)
Other comprehensive loss:				
Foreign currency translation adjustments	225	(4,403)	(64)	(5,051)
Transfer of cumulative translation adjustment for the substantial liquidation of foreign subsidiaries	—	—	5,272	—
Unrealized gain (loss) on available-for-sale securities	67	(51)	(182)	(77)
Other comprehensive income (loss):	292	(4,454)	5,026	(5,128)
Total comprehensive loss	$(52,436)	$(77,345)	$(17,449)	$(146,309)

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(Unaudited)

(In thousands)

	Six Months Ended June 30,	
	2018	2017
Operating activities:		
Net loss	$(22,475)	$(141,181)
Adjustments to reconcile net loss to net cash used in operating activities:		
Gain from sale of priority review vouchers	(170,322)	—
Stock-based compensation	38,360	31,293
Amortization of premium (discount) on investment securities, net	(913)	1,051
Depreciation and amortization	12,240	2,216
Foreign currency remeasurement (gain) loss	5,846	(4,604)
Other	(145)	—
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(22,382)	469
Other assets	(102)	101
Accounts payable	1,376	6,353
Accrued liabilities and other liabilities	(7,039)	(5,725)
Net cash used in operating activities	(165,556)	(110,027)
Investing activities:		
Purchase of property and equipment	(1,852)	(861)
Proceeds from sale of priority review vouchers	170,322	—
Purchase of investments	(408,683)	(207,208)
Proceeds from the sale of investments	4,954	21,642
Proceeds from maturities of investments	112,162	154,730
Net cash used in investing activities	(123,097)	(31,697)
Financing activities:		
Proceeds from issuance of common stock in connection with a public offering, net	270,969	—
Proceeds from issuance of common stock in connection with at-the-market offering, net	11,789	67,616
Proceeds from issuance of common stock from equity awards, net	16,829	2,879
Net cash provided by financing activities	299,587	70,495
Effect of exchange rate changes on cash	(647)	(361)
Net increase (decrease) in cash, cash equivalents and restricted cash	10,287	(71,590)
Cash, cash equivalents and restricted cash at beginning of period	103,041	164,607
Cash, cash equivalents and restricted cash at end of period	$113,328	$93,017

See accompanying notes.

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ULTRAGENYX PHARMACEUTICAL INC.

Notes to Condensed Consolidated Financial Statements

1. Organization

Ultragenyx Pharmaceutical Inc. (the Company) is a biopharmaceutical company and was incorporated in California on April 22, 2010. The Company subsequently reincorporated in the state of Delaware in June 2011.

The Company is focused on the identification, acquisition, development, and commercialization of novel products for the treatment of rare and ultra-rare diseases, with a focus on serious, debilitating genetic diseases. The Company has two approved therapies. Crysvita® (burosumab) is approved by the U.S. Food and Drug Administration (FDA) for the treatment of X-linked hypophosphatemia (XLH) in adult and pediatric patients one year of age and older, and has received European conditional marketing authorization for the treatment of XLH with radiographic evidence of bone disease in children 1 year of age and older and adolescents with growing skeletons. The Company has also received FDA approval for Mepsevii™ (vestronidase alfa), the first medicine approved for the treatment of children and adults with MPS VII, also known as Sly syndrome.

In addition to the approved treatments for XLH and MPS VII, the Company has six ongoing clinical development programs. Crysvita is being studied for the treatment of tumor induced osteomalacia (TIO), a rare disease that impairs bone mineralization. UX007 is being studied in patients with glucose transporter type-1 deficiency syndrome (Glut1 DS), a brain energy deficiency, who are experiencing movement disorders; it is also being studied in patients severely affected by long-chain fatty acid oxidation disorders (LC-FAOD), a genetic disorder in which the body is unable to convert long chain fatty acids into energy. The company has three gene therapy pipeline candidates: DTX 301 is an adeno-associated virus 8 (AAV8) gene therapy product candidate in development for the treatment of patients with ornithine transcarbamylase (OTC) deficiency, the most common urea cycle disorder; DTX 401 is an AAV8 gene therapy product candidate for the treatment of patients with glycogen storage disease type Ia (GSDIa); and DTX201 is an FVIII gene therapy clinical candidate for the treatment of hemophilia A. The Company operates as one reportable segment.

The Company has sustained operating losses and expects such annual losses to continue over the next several years. The Company’s ultimate success depends on the outcome of its research and development activities. Management expects to incur additional losses in the future to conduct product research and development and recognizes the need to raise additional capital to fully implement its business plan. Through June 30, 2018, the Company has relied primarily on the proceeds from equity offerings to finance its operations.

The Company intends to raise additional capital through the issuance of equity, borrowings, or strategic alliances with partner companies. However, if such financing is not available at adequate levels, the Company will need to reevaluate its operating plans.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements include the amounts of the Company and our wholly-owned subsidiaries and have been prepared in accordance with U.S. generally accepted accounting principles (GAAP) for interim financial information and in accordance with the instructions to Form 10-Q and Rule 10-01 of Regulation S-X. Accordingly, they do not include all of the information and footnotes required by GAAP for complete financial statements. The unaudited interim consolidated financial statements have been prepared on the

same basis as the annual financial statements. In the opinion of management, the accompanying unaudited condensed consolidated financial statements reflect all adjustments (consisting only of normal recurring adjustments) considered necessary for a fair presentation. These financial statements should be read in conjunction with the audited financial statements and notes thereto for the preceding fiscal year contained in the Company's Annual Report on Form 10-K filed on February 21, 2018 with the United States Securities and Exchange Commission (SEC).

The results of operations for the three and six months ended June 30, 2018 are not necessarily indicative of the results to be expected for the year ending December 31, 2018. The condensed consolidated balance sheet as of December 31, 2017 has been derived from audited financial statements at that date, but does not include all of the information required by GAAP for complete financial statements.

Use of Estimates

The accompanying consolidated financial statements have been prepared in accordance with GAAP. The preparation of the consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent liabilities and the reported amounts of expenses in the consolidated financial statements and the accompanying notes. On an ongoing basis, management evaluates its estimates, including those related to sales return reserves, clinical trial accruals, fair value of assets and liabilities, income taxes, and stock-based compensation. Management bases its estimates on historical experience and on various other market-specific and relevant assumptions that management believes to be reasonable under the circumstances. Actual results could differ from those estimates.

Cash, Cash Equivalents and Restricted Cash

Restricted cash primarily consists of money market accounts as collateral for the Company's obligations under its facility leases.

In November 2016, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) 2016-18, Statement of Cash Flows (Topic 230): Restricted Cash, which requires restricted cash to be presented with cash and cash equivalents on the statement of cash flows and disclosure of how the statement of cash flows reconciles to the balance sheet if restricted cash is shown separately from cash and cash equivalents on the balance sheet. The Company adopted the standard as of January 1, 2018 on a retrospective basis, wherein the statement of cash flow of each period presented was adjusted to reflect the effects of applying the new guidance. The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the consolidated balance sheets that sum to the total of the same such amounts shown in the consolidated statement of cash flows (in thousands):

	June 30, 2018	June 30, 2017
Cash and cash equivalents	$110,854	$90,751
Restricted cash included in prepaid expenses and other current assets	652	461
Restricted cash included in other assets	1,822	1,805
Total cash, cash equivalents, and restricted cash shown in the statements of cash flows	$113,328	$93,017

Revenue Recognition

Collaboration and license revenue

The Company has certain license and collaboration agreements that are within the scope of Accounting Standards Codification (ASC) 808, Collaborative Agreements, which provides guidance on the presentation and disclosure of collaborative arrangements. Generally, the classification of the transactions under the collaborative arrangements is determined based on the nature of contractual terms of the arrangement, along with the nature of the operations of the participants. The Company records its share of collaboration revenue, net of transfer pricing related to net sales in the period in which such sales occur, if the Company is considered as an agent in the arrangement. The Company is considered an agent when the collaboration partner controls the product before transfer to the customers and has the ability to direct the use of and obtain substantially all of the remaining benefits from the product. Funding received related to research and development services and commercialization costs are classified as a reduction of research and development expenses and selling, general and administrative expenses, respectively in the consolidated statement of operations, because the provision of such services for collaborative partners are not considered to be part of the Company's ongoing major or central operations.

The Company also receives royalty revenues under certain of the Company's license or collaboration agreements in exchange for license of intellectual property. If the Company does not have any future performance obligations for these license or collaboration agreements, royalty revenue is recorded as the underlying sales occur.

In order to record its results of operations, the Company utilizes certain information from its collaboration partners , including revenue from the sale of the product, associated reserves on revenue, and costs incurred for development

and sales activities. For the periods covered in the financial statements presented, there have been no significant or material changes to prior period estimates of revenues and expenses.

The terms of the Company's collaboration agreements may contain multiple performance obligations, which may include licenses and research and development activities. The Company evaluates these agreements under ASC 606, Revenue from Contract with Customers, to determine the distinct performance obligations. The Company analogizes to ASC 606 for the accounting for distinct performance obligations for which there is a customer relationship. Prior to recognizing revenue, the Company makes estimates of the transaction price, including variable consideration that is subject to a constraint. Amounts of variable consideration are included in the transaction price to the extent that it is probable that a significant reversal in the amount of cumulative revenue recognized will not occur and when the uncertainty associated with the variable consideration is subsequently resolved. Variable consideration may include nonrefundable upfront license fees, payments for research and development activities, reimbursement of certain third-party costs, payments based upon the achievement of specified milestones, and royalty payments based on product sales derived from the collaboration.

If there are multiple distinct performance obligations, the Company allocates the transaction price to each distinct performance obligation based on its relative standalone selling price. The standalone selling price is generally determined based on the prices charged to customers or using expected cost plus margin. Revenue is recognized by measuring the progress toward complete satisfaction of the performance obligations using an input measure.

Product sales

The Company sells Mepsevii through a limited number of distributors. Under ASC 606, revenue from product sales is recognized at the point in time when the delivery is made and when title and risk of loss transfers to these distributors. The Company also recognizes revenue from sales of certain products on a “named patient” basis, which are allowed in certain countries prior to the commercial approval of the product. Prior to recognizing revenue, the Company makes estimates of the transaction price, including any variable consideration that is subject to a constraint. Amounts of variable consideration are included in the transaction price to the extent that it is probable that a significant reversal in the amount of cumulative revenue recognized will not occur when the uncertainty associated with the variable consideration is subsequently resolved. Product sales are recorded net of estimated government-mandated rebates and chargebacks, estimated product returns, and other deductions.

Provisions for returns and other adjustments are provided for in the period the related revenue is recorded, as estimated by management. Limited historical data is available for use in developing estimates of the amount of the reduction for reserve in gross revenue. The estimates applied are periodically reviewed and adjusted as necessary.

Recently Issued Accounting Pronouncements

In February 2016, the FASB issued ASU No. 2016-02, Leases, which requires an entity that is a lessee to record a right of use asset and a corresponding lease liability on the balance sheet for all leases with terms longer than 12 months. This guidance also requires disclosures about the amount, timing, and uncertainty of cash flows arising from leases. This guidance is effective for annual reporting periods beginning after December 15, 2018, and interim periods within those annual periods, using a modified retrospective approach, and early adoption is permitted. The Company is evaluating the effect that this guidance will have on its Consolidated Financial Statements and related disclosures.

3. Financial Instruments

Financial assets and liabilities are recorded at fair value. The carrying amount of certain financial instruments, including cash and cash equivalents, accounts payable and accrued liabilities approximate fair value due to their relatively short maturities. Assets and liabilities recorded at fair value on a recurring basis in the balance sheets are categorized based upon the level of judgment associated with the inputs used to measure their fair values. Fair value is defined as the exchange price that would be received for an asset or an exit price that would be paid to transfer a liability in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. The authoritative guidance on fair value measurements establishes a three-tier fair value hierarchy for disclosure of fair value measurements as follows:

Level 1—Inputs are unadjusted, quoted prices in active markets for identical assets or liabilities at the measurement date;

Level 2—Inputs are observable, unadjusted quoted prices in active markets for similar assets or liabilities, unadjusted quoted prices for identical or similar assets or liabilities in markets that are not active, or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the related assets or liabilities; and

Level 3—Unobservable inputs that are significant to the measurement of the fair value of the assets or liabilities that are supported by little or no market data.

The following tables set forth the fair value of the Company’s financial assets remeasured on a recurring basis based on the three-tier fair value hierarchy (in thousands):

	Fair Value Hierarchy	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Money market funds	Level 1	$ 94,565	$ —	$ —	$ 94,565
Time deposits	Level 2	10,000	—	—	10,000
Corporate bonds	Level 2	160,606	—	(311)	160,295
Commercial paper	Level 2	71,708	—	—	71,708
Asset-backed securities	Level 2	30,210	—	(35)	30,175
U.S. Government Treasury and agency securities	Level 2	174,316	1	(217)	174,100
Total		$ 541,405	$ 1	$ (563)	$ 540,843

	December 31, 2017			
	Fair Value Hierarchy	Amortized Cost	Gross Unrealized Gains	Losses	Estimated Fair Value
Money market funds	Level 1	$ 79,670	$ —	$ —	$ 79,670
Corporate bonds	Level 2	39,330	—	(90)	39,240
U.S. Government Treasury and agency securities	Level 2	105,029	—	(290)	104,739
Total		$ 224,029	$ —	$ (380)	$ 223,649

At June 30, 2018, the remaining contractual maturities of available-for-sale securities were at or less than one year. There have been no significant realized gains or losses on available-for-sale securities for the periods presented. All marketable securities with unrealized losses at June 30, 2018 have a loss that was considered to be temporary in nature. The Company does not intend to sell the investments that are in an unrealized loss position before recovery of their amortized cost basis.

4. Balance Sheet Components

Inventory

Inventory consists of the following (in thousands):

	June 30, 2018	December 31, 2017
Work-in-progress	$ 2,880	$ 737
Finished goods	462	20
Total inventory	$ 3,342	$ 757

Accrued Liabilities

Accrued liabilities consist of the following (in thousands):

	June 30, 2018	December 31, 2017
Research and clinical study expenses	$ 22,761	$ 17,141
Payroll and related expenses	23,479	26,527
Repayment liability under collaboration agreement	—	3,681
Contract liabilities	1,244	5,986
Other	8,251	8,793
Total accrued liabilities	$ 55,735	$ 62,128

5. Revenue

The following table disaggregates total revenues from external customers by collaboration and license revenue and product sales (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Collaboration and license revenue:				
KHK (Crysvita)	$1,577	$ —	$1,592	$ —
Bayer	8,942	—	18,289	—
Total collaboration and license revenue	10,519	—	19,881	—
Product sales:				
Crysvita	26	—	26	—
Mepsevii	2,007	—	3,126	—
UX007	242	—		