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Industry SnapShots

Up to date business intelligence reports covering developments in the world's fastest growing industries

UNITED STATES BIOTECHNOLOGY

2 August 2016

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- **pharmabiz - Icagen buys Nanion's SyncroPatch384 high-throughput electrophysiology platform - 29/7/2016**
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- **Fierce Biotech - Celgene affirms Juno CAR-T deal as key to long-term growth - 28/7/2016**
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Industry SnapShots

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News and Commentary

pharmabiz - US FDA grants Fast Track designation to Shire's SHP626 to treat adults who have NASH with liver fibrosis - 1/8/2016

FDA has granted Fast Track designation for Shire's SHP626 for an investigational treatment of adults who have nonalcoholic steatohepatitis with liver fibrosis.

For the complete story see:
http://www.pharmabiz.com/NewsDetails.aspx?aid=96606&sid=2

pharmabiz - Icagen buys Nanion's SyncroPatch384 high-throughput electrophysiology platform - 29/7/2016

Icagen, Inc. and Nanion announced that Icagen has purchased a SyncroPatch384 high-throughput electrophysiology instrument.

For the complete story see:
http://www.pharmabiz.com/NewsDetails.aspx?aid=96552&sid=2

Fierce Biotech - Celgene affirms Juno CAR-T deal as key to long-term growth - 28/7/2016

Celgene highlighted a handful of immuno-oncology deals that are driving its long-term R&D prospects.

For the complete story see:
http://www.fiercebiotech.com/biotech/celgene-affirms-juno-car-t-deal-as-key-to-long-term-growth

Washington Post - Major global partnership to speed antibiotic development launched - 28/7/2016

CARB-X will create one of the world's largest public-private partnerships focused on preclinical discovery and development of new antimicrobial products.

For the complete story see:
https://www.washingtonpost.com/news/to-your-health/wp/2016/07/28/major-global-partnership-to-speed-antibiotic-development-launched/

PR Newswire - S.C. Department of Commerce partners with SCBIO - 28/7/2016

The partnership will identify obstacles for existing companies and opportunities to create more competitive advantages.

For the complete story see:
http://www.prnewswire.com/news-releases/sc-department-of-commerce-partners-with-scbio-300305623.html

pharmabiz - X-Chem, AbbVie ink multi-target drug discovery partnership - 28/7/2016

The collaboration is focused on the discovery and development of novel treatments for diseases in oncology and immunology.

For the complete story see:
http://www.pharmabiz.com/NewsDetails.aspx?aid=96531&sid=2

WSJ - Vertex Pharma Loss Narrows as Cystic Fibrosis Drug Sales Grow - 27/7/2016

Company also affirms 2016 guidance for Orkambi and Kalydeco treatments.

For the complete story see:
http://www.wsj.com/articles/vertex-pharma-loss-narrows-as-cystic-fibrosis-drug-sales-grow-1469652198

PMLiVE - Celgene disappointed as Revlimid fails lymphoma trial - 27/7/2016

Study shows improvement in progression-free survival but no overall survival rate benefit.

For the complete story see:
http://www.pmlive.com/pharma_news/celgene_disappointed_as_revlimid_fails_lymphoma_trial_1082856

eNCA - Americans fear biotechnology advances - 26/7/2016

Futuristic technologies that promise to improve people's strength and smarts have raised more concern than enthusiasm among Americans.

For the complete story see:
https://www.enca.com/technology/americans-fear-biotechnology-advances-poll

Media Releases

Shire Pharmaceuticals (NASDAQ: SHPG) - Shire's SHP626 (Volixibat) Receives FDA Fast Track Designation for an Investigational Treatment for Adults who have Nonalcoholic Steatohepatitis (NASH) with Liver Fibrosis – 29/7/2016

July 29, 2016

Lexington, Mass. – July 29, 2016 – Shire plc (LSE: SHP, NASDAQ: SHPG) today announced that the United States Food and Drug Administration (FDA) has granted Fast Track designation for SHP626 (volixibat) for an investigational treatment of adults who have nonalcoholic steatohepatitis (NASH) with liver fibrosis. Shire is developing SHP626 as a once daily, orally-administered inhibitor of the apical sodium dependent bile acid transporter (ASBT), a protein which is primarily responsible for recycling bile acids from the intestine to the liver. NASH is a serious, chronic liver disease for which there are currently no approved drugs.

"Shire's development plan for SHP626 is designed to address the unmet need in the treatment of adult patients who have NASH with liver fibrosis," said Philip J. Vickers, Ph.D., Head of R&D, Shire. "This Fast Track designation is further recognition of the critical need to develop new, effective therapeutic options for patients with this serious condition." The FDA Fast Track Designation for SHP626 in NASH was supported by preclinical and Phase 1 studies. The FDA's Fast Track is a process designed to facilitate the development, and expedite the review of drugs to treat serious conditions and fill an unmet medical need. However, it does not guarantee that the FDA will ultimately approve SHP626 for NASH or the timing of any such approval.

Shire will initiate its Phase 2 trial with SHP626 as a randomized, placebo-controlled, double-blind study to evaluate the safety, tolerability and efficacy of three doses of volixibat over 48-weeks in adult patients with NASH. The Phase 2 study will be conducted in the U.S., Canada and the United Kingdom.

SHP626 has been evaluated in preclinical and Phase 1 studies, in which the safety, tolerability and preliminary activity of SHP626 compared to placebo in healthy volunteers, as well as in overweight and obese volunteers, was assessed. The most common adverse events occurring in Phase 1 trials of SHP626 were gastrointestinal in nature, predominantly diarrhea. While this occurred in most patients, it was not considered serious. There was one serious adverse event reported that was considered related to SHP626, alanine aminotransferase elevation, that led to discontinuation of drug.

About Nonalcoholic Steatohepatitis (NASH)

NASH is a type of nonalcoholic fatty liver disease (NAFLD), characterized by inflammation and the accumulation of fat in the liver, for which there are currently no approved drugs. It can be severe and lead to fibrosis, cirrhosis, liver failure and liver cancer. There is a steady rise in the prevalence of NASH in the U.S. and globally, and the disease is typically associated with obesity, type 2 diabetes, hypertension, high cholesterol and triglycerides. NASH is currently the second leading cause of liver transplantation in adults in the U.S., and is estimated to become the leading cause for liver transplantation if the current trajectory continues.

https://www.shire.com/newsroom/2016/july/7zve23

Alexion Pharmaceuticals (NASDAQ: ALXN) - Alexion Reports Second Quarter 2016 Results – 28/7/2016

- Total Revenues of $753 Million; Increased 18 Percent Year-on-Year; 23 Percent Volume Increase Year-on-Year-

- Soliris® (eculizumab) Revenue Growth Driven by Steady Number of New Patients with PNH and aHUS Treated Globally -

- Strong Strensiq® (asfotase alfa) Launch Continues in Initial Countries -

- Kanuma® (sebelipase alfa) Launch Progresses with Newly Identified Patients Starting on Treatment -

- Eculizumab Phase 3 REGAIN Data in Refractory gMG Presented at the ICNMD Congress -

- ALXN1210 Phase 1/2 Data Showed Rapid and Sustained Reductions in LDH in All Patients with PNH Treated with Once-Monthly Dosing -

- SBC-103 Phase 1/2 Data on MRI and Neurocognitive Assessments Consistent With Potential Dose-Dependent Disease Stabilization at Six Months in Patients with MPS IIIB -

- GAAP EPS of $0.51 Per Share; Non-GAAP EPS of $1.13 Per Share, Which Reflects a Reduction of $0.12 Per Share Attributable to the Modification of Reported Non-GAAP Income Tax Expense –

Thursday, July 28, 2016 6:30 am EDT

NEW HAVEN, Conn. - (BUSINESS WIRE) - Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN) today announced financial results for the second quarter of 2016. Total revenues grew to $753 million, an 18 percent increase, compared to $636 million for the same period in 2015. In the second quarter, the negative impact of currency on total revenue was 3 percent or $18 million, net of hedging activities, compared to the same quarter last year. On a GAAP basis, diluted earnings per share (EPS) for the second quarter of 2016 was $0.51 per share, compared to $0.83 per share in the second quarter of 2015. Non-GAAP diluted EPS for the second quarter 2016 was $1.13 per share, reflecting a reduction of $0.12 per share attributable to the modification of reported non-GAAP income tax expense; prior to this modification non-GAAP diluted EPS would have been reported at $1.25 per share (Table 2). Non-GAAP diluted EPS was $1.30 per share in the second quarter 2015, reflecting a reduction of $0.14 per share attributable to the tax modification.

Alexion has modified the definition of its non-GAAP income tax expense to align with the Compliance & Disclosure Interpretations (C&DIs) issued by the U.S. Securities and Exchange Commission (SEC) on May 17, 2016, and has reflected this modification in 2015 and 2016 non-GAAP interim period results. Alexion's modified definition no longer includes the cash tax benefits the Company realizes during the year from net operating losses and income tax credits, and now includes other deferred taxes. The modification does not change the amount of cash taxes the Company will pay in 2016, or in the future, or have any impact on cash flow. A reconciliation of GAAP to non-GAAP financial results (Table 2) and supplemental effective tax rate information for financial guidance (Table 6) are provided later in the press release.

"In Q2 2016, we delivered strong financial performance as we served an increasing number of patients with PNH, aHUS, HPP and LAL-D. We are pleased with the sustained growth in our core Soliris business, the strong launch of Strensiq, and the continued progress with our Kanuma launch," said David Hallal, Chief Executive Officer of Alexion. "In the second half of 2016, we will continue to leverage our rare disease expertise to reach more patients with Soliris, Strensiq and Kanuma while advancing multiple milestones in our robust pipeline."

Second Quarter 2016 Financial Highlights

- Soliris® (eculizumab) net product sales were $701 million, compared to $636 million in Q2 2015, representing a 10 percent increase. Soliris volume increased 15 percent year-on-year.
- Strensiq® (asfotase alfa) net product sales were $45 million.
- Kanuma® (sebelipase alfa) net product sales were $6 million.
- GAAP R&D expense was $179 million, compared to $132 million in the same quarter last year. Non-GAAP R&D expense was $165 million, compared to $117 million in the same quarter last year.
- GAAP SG&A expense was $232 million, compared to $221 million in the same quarter last year. Non-GAAP SG&A expense was $200 million, compared to $169 million in the same quarter last year.
- GAAP diluted EPS was $0.51 per share, compared to $0.83 per share in the same quarter last year. Non-GAAP diluted EPS was $1.13 per share, reflecting a reduction of $0.12 per share attributable to the

modification of reported non-GAAP income tax expense, compared to $1.30 per share, reflecting a reduction of $0.14 per share attributable to the modification of non-GAAP income tax expense in the same quarter last year. GAAP and non-GAAP EPS in the second quarter of 2016 includes the impact of a full quarter of Synageva operations, shares issued for the acquisition and interest expense on related borrowings.

Product and Pipeline Updates

Complement Portfolio

- Eculizumab- Refractory Generalized Myasthenia Gravis (gMG): Data from the REGAIN study, a single, multinational, placebo-controlled Phase 3 trial of eculizumab in patients with refractory gMG, were presented at the International Congress on Neuromuscular Diseases (ICNMD) meeting. Alexion expects to provide an update on discussions with regulators by the end of the year.
- Eculizumab- Relapsing Neuromyelitis Optica Spectrum Disorder (NMOSD): Alexion expects to complete enrollment this year in the PREVENT study, a single, multinational, placebo-controlled Phase 3 trial of eculizumab in patients with relapsing NMOSD.
- Eculizumab- Delayed Graft Function (DGF): Enrollment is complete in the PROTECT study, a single, multinational, placebo-controlled Phase 3 trial of eculizumab in the prevention of DGF, and data are expected in the second half of 2016.
- ALXN1210: New data from the Phase 1/2 study of ALXN1210, a highly innovative longer-acting C5 antibody, in patients with paroxysmal nocturnal hemoglobinuria (PNH) were presented at the European Hematology Association (EHA) Congress. Alexion expects to present additional PNH data later this year. Alexion also expects to initiate a clinical program with ALXN1210 in patients with atypical hemolytic uremic syndrome (aHUS) later this year. The European Commission granted Orphan Drug Designation (ODD) to ALXN1210 for the treatment of patients with PNH.
- ALXN1007: New data from the Phase 2 study of ALXN1007, a complement inhibitor that targets C5a, in patients with graft-versus-host disease involving the lower gastrointestinal tract (GI-GVHD) were presented at EHA and Alexion is now evaluating higher doses of ALXN1007 in patients with GI-GVHD.

Metabolic Portfolio

- SBC-103: New Phase 1/2 data of SBC-103, a recombinant form of the NAGLU enzyme, in patients with mucopolysaccharidosis IIIB, or MPS IIIB, were presented at the International Symposium on MPS and Related Diseases meeting. Alexion has now completed the planned dose escalation, with all patients now randomized to either a 5 mg/kg or 10 mg/kg dose. A natural history study to characterize the course of disease progression in patients with MPS IIIB is ongoing.
- cPMP Replacement Therapy (ALXN1101): Alexion is enrolling patients in a pivotal study to evaluate ALXN1101 in neonates with Molybdenum Cofactor Deficiency (MoCD) Type A. A study to characterize the natural history of MoCD type A was completed in Q2.

Preclinical Portfolio

- Alexion has more than 30 diverse preclinical programs across a range of therapeutic modalities, with four of these programs expected to enter the clinic in 2016.

2016 Financial Guidance

Alexion is reiterating its total revenue and Soliris guidance ranges provided on the first quarter of 2016 earnings call on April 28, 2016, and based on the strength of the Strensiq launch is increasing its Metabolic revenue guidance to $200 to $220 million. Alexion is reiterating its non-GAAP operating expense guidance and is updating its non-GAAP tax rate and non-GAAP EPS guidance. Alexion is also issuing 2016 GAAP financial guidance.

2016 financial guidance is as follows:

	GAAP Guidance	Updated Non-GAAP Guidance	Prior Non-GAAP Guidance
Total revenues	$3,050 to $3,100 million	$3,050 to $3,100 million	Low end of $3,050 to $3,100 million
Soliris revenues	$2,835 to $2,875 million	$2,835 to $2,875 million	$2,835 to $2,875 million
Metabolic revenues	$200 to $220 million	$200 to $220 million	$180 to $200 million
Cost of sales	8% to 9%	8% to 9%	8% to 9%
Research and development expense	$708 to $779 million	High end of $650 to $680 million	High end of $650 to $680 million
Selling, general and administrative expense	$883 to $935 million	High end of $760 to $790 million	High end of $760 to $790 million
Interest expense	$100 million	$100 million	$100 million
Effective tax rate	32% to 34%	15.5% to 16.5% (1)	7% to 8%
Earnings per share	$1.91 to $2.26	$4.50 to $4.65	Low end of $5.00 to $5.20
Diluted shares outstanding	228 million	230 million	230 million

http://news.alexionpharma.com/press-release/financial-news/alexion-reports-second-quarter-2016-results

Celgene Corporation (NASDAQ: CELG) - Celgene Reports Second Quarter 2016 Operating and Financial Results – 28/7/2016

- Net Product Sales $2.74B in Q2:16: Increased 22% Y/Y
- REVLIMID® Net Product Sales $1.7B in Q2:16; Increased 18% Y/Y
- 2016 Guidance Updated: REVLIMID® and Total Net Product Sales; EPS

Jul 28, 2016

SUMMIT, N.J. - (BUSINESS WIRE) - Celgene Corporation (NASDAQ:CELG) reported net product sales of $2,745 million for the second quarter of 2016, a 22 percent increase from the same period in 2015. Net product sales growth includes a 1 percent negative impact from currency exchange effects. Second quarter total revenue increased 21 percent to $2,754 million compared to $2,278 million in the second quarter of 2015.

Net income for the second quarter of 2016 based on U.S. GAAP (Generally Accepted Accounting Principles), was $598 million or $0.75 per diluted share compared to $356 million or $0.43 per diluted share in the second quarter of 2015. Adjusted net income for the second quarter of 2016 was$1,152 million or $1.44 per diluted share compared to $1,019 million or $1.23 per diluted share for the second quarter of 2015.

"Our first-half 2016 operating results were outstanding and we are pleased with the progress made advancing many key corporate objectives," said Mark J. Alles, Chief Executive Officer of Celgene Corporation. "This strong momentum increases our confidence in our near- and longer-term outlook as we continue to invest in innovative research and the development of transformational therapies for patients worldwide."

Second Quarter 2016 Financial Highlights

Unless otherwise stated, all comparisons are for the second quarter of 2016 compared to the second quarter of 2015. The adjusted operating expense categories presented below exclude share-based employee compensation expense, upfront collaboration expense and a litigation-related loss contingency accrual expense. Please see the attached Reconciliation of GAAP to Adjusted Net Income for further information.

Net Product Sales Performance

- REVLIMID® sales for the second quarter increased 18 percent year-over-year to $1,701 million and were driven by new patient market share gains and increased duration. U.S. sales of $1,080 million and international sales of $621 million increased 24 percent and 9 percent year-over-year, respectively.
- POMALYST®/IMNOVID® sales for the second quarter were $318 million, an increase of 35 percent year-over-year. U.S. sales were $185 million and international sales were $133 million, an increase of 29 percent and 46 percent year-over-year, respectively. POMALYST®/IMNOVID® sales grew due to increased volume from duration gains.
- ABRAXANE® sales for the second quarter were $249 million, a 2 percent increase year-over-year. U.S. sales of $175 million increased 3 percent year-over-year. International sales were $74 million.
- OTEZLA® sales for the second quarter were $242 million, a 170 percent increase year-over-year. U.S. sales were $217 million and international sales were $25 million. Sales were driven by market share gains and increased prescriber adoption.
- In the second quarter, all other product sales, which include THALOMID®, ISTODAX®, VIDAZA® and an authorized generic version of VIDAZA® drug product in the U.S., were $235 million compared to $242 million in the second quarter of 2015.

Research and Development (R&D)

On a GAAP basis, R&D expenses were $949 million for the second quarter of 2016 compared to $1,110 million for the same period in 2015. The change was primarily driven by a decrease in upfront collaboration expenses compared to the previous year, partially offset by early research and clinical trial activity related to the acquisitions of Receptos, Inc. and Quanticel Pharmaceuticals, Inc. that closed in the second half of 2015. Adjusted R&D expenses were $601 million for the second quarter of 2016 compared to $477 million for the second quarter of 2015. Adjusted R&D does not include upfront collaboration expenses but does reflect the increase in early research and clinical trial activity.

Selling, General, and Administrative (SG&A)

On a GAAP basis, SG&A expenses were $732 million for the second quarter of 2016 compared to $617 million for the same period in 2015. The increase was primarily due to a loss contingency accrual expense of $100 million related to a contractual dispute. Adjusted SG&A expenses were$547 million for the second quarter of 2016 compared to $541 million for the second quarter of 2015.

Cash, Cash Equivalents, and Marketable Securities

Operating cash flow was $936 million in the second quarter of 2016. Celgene ended the quarter with approximately $6.4 billion in cash, cash equivalents and marketable securities.

In the second quarter of 2016, Celgene purchased approximately 3.4 million of its shares at a total cost of approximately $343 million. In June 2016, the share repurchase authorization was increased by an additional authorization of $3 billion. As of June 30, 2016, the Company had approximately $5.1 billion remaining under the stock repurchase program.

2016 Guidance Updated

	Previous 2016 Guidance	Updated 2016 Guidance
Net Product Sales		
Total	$10.75B-$11.0B	Approximately $11.0B
REVLIMID®	Approximately $6.7B	Approximately $6.8B
GAAP diluted EPS	$4.26 to $4.56	$3.82 to $4.05
Adjusted diluted EPS	$5.60 to $5.70	$5.70 to $5.75

GAAP operating margin	Approximately 42%	Approximately 37%
Adjusted operating margin	Approximately 53.5%	Approximately 54.0%
Weighted average diluted shares	811M	806M

Net product sales guidance for POMALYST®/IMNOVID®, ABRAXANE® and OTEZLA® remain unchanged.

Product and Pipeline Updates

Hematology/Oncology

- At the American Society of Clinical Oncology (ASCO) meeting in June, pooled data from a meta-analysis of overall survival (OS) in multiple myeloma patients receiving REVLIMID® as maintenance treatment following autologous stem-cell transplant were presented. An application was submitted to the European Medicines Agency (EMA) in early June for the review of REVLIMID® as maintenance treatment in newly diagnosed multiple myeloma (NDMM) patients after receiving an autologous stem-cell transplant. A decision on the application is expected in 2017. A submission in the U.S. is expected in the second half of 2016.
- In July, the European Commission (EC) approved REVLIMID® for the treatment of adult patients with relapsed or refractory mantle cell lymphoma. REVLIMID® is approved in the U.S. for the treatment of mantle cell lymphoma after relapse or progression on two prior therapies.
- In June, the U.S. product insert for POMALYST® was updated to include data from a pooled pharmacokinetics analysis of patients with relapsed and/or refractory multiple myeloma (RRMM) and impaired renal function. In Europe, the Committee for Medicinal Products for Human Use (CHMP) granted a positive opinion for IMNOVID® based on the same data. The EC decision is expected in the third quarter.
- In July, Celgene disclosed the top-line results of the phase III REMARC trial evaluating REVLIMID® as maintenance therapy compared with placebo in patients with diffuse large B-cell lymphoma responding to treatment with rituximab in combination with standard chemotherapy. The full data set will be presented at a future medical congress.
- In July, Celgene's partner Juno Therapeutics provided preliminary data from the ongoing phase I trial with JCAR017 in patients with adult non-Hodgkin lymphoma (NHL). In ten patients evaluable for efficacy, an overall response rate of 80 percent and a complete response rate of 70 percent were seen. In thirteen patients evaluable for safety, the rate of severe neurotoxicity was 15 percent and the rate of cytokine release syndrome was zero percent. An update of the trial data is expected later in the year.
- The FUSION™ program evaluating durvalumab in hematological malignancies continues to advance with six early-stage trials enrolling. The trials are evaluating durvalumab as a single agent or in combination with novel agents in NDMM, RRMM, myelodysplastic syndromes, acute myeloid leukemia, NHL and chronic lymphocytic leukemia.
- A phase II trial with CC-486 in combination with pembrolizumab in previously treated locally advanced or metastatic non-small cell lung cancer completed enrollment in the second quarter.

Inflammation & Immunology

- Long-term data from the PALACE program evaluating OTEZLA® in moderate-to-severe psoriatic arthritis were presented at the EuropeanLeague Against Rheumatism (EULAR) meeting in June. Included was three-year pooled efficacy and safety data from the phase III PALACE program, as well as pooled data on fatigue, HAQ-DI and BASDAI from PALACE 1-3.
- The phase II proof-of-concept trial evaluating OTEZLA® in atopic dermatitis has completed. Celgene is evaluating the data to determine next steps. The data will be published at a later date.
- In May, the phase II TOUCHSTONE trial evaluating ozanimod induction and maintenance in patients with moderate-to-severe ulcerative colitis was published in *The New England Journal of Medicine*. Histologic data from the phase II TOUCHSTONE trial were presented at the Digestive Disease Week meeting in May. The phase III TRUE NORTH trial evaluating ozanimod in patients with moderate-to-severe ulcerative colitis continues to enroll with data expected in 2018.

- The registration-enabling endoscopy trial (CD-001) with GED-0301 in patients with active Crohn's disease completed enrollment. Top-line data from the 12-week portion of the trial is expected in the second half of 2016.

Business Update

- In July, Celgene announced a strategic collaboration with Jounce Therapeutics, Inc. The collaboration includes options on Jounce's lead product candidate, JTX-2011, targeting ICOS (the Inducible T cell CO-Stimulator), and up to four early-stage programs to be selected from a defined pool of B cell, T regulatory cell and tumor-associated macrophage targets emerging from Jounce's research platform, and an additional option on a Jounce checkpoint immuno-oncology program.
- In May, Celgene and Agios Pharmaceuticals, Inc. entered into a new global strategic collaboration for the discovery, development and commercialization of novel metabolic immuno-oncology therapies based on Agios' innovative cellular metabolism research platform. In addition, Celgene transferred global development and commercialization rights to the AG-120 program to Agios.

http://ir.celgene.com/releasedetail.cfm?ReleaseID=981642

Amgen (NASDAQ: AMGN) - Amgen Reports Second Quarter 2016 Financial Results – 27/7/2016

THOUSAND OAKS, Calif., July 27, 2016 /PRNewswire/ - Amgen (NASDAQ:AMGN) today announced financial results for the second quarter of 2016. Key results include:

- Revenues increased 6 percent versus the second quarter of 2015 to $5.7 billion.
  - Product sales grew 5 percent driven by Enbrel® (etanercept), Prolia® (denosumab), KYPROLIS® (carfilzomib) and XGEVA® (denosumab).
- GAAP earnings per share (EPS) increased 15 percent to $2.47 driven by higher revenues and higher operating margins.
  - GAAP operating income increased 15 percent to $2,380 million and GAAP operating margin improved by 3.8 percentage points to 43.5 percent.
- Non-GAAP EPS increased 11 percent to $2.84 driven by higher revenues and higher operating margins.
  - Non-GAAP operating income increased 10 percent to $2,812 million and non-GAAP operating margin improved by 2.6 percentage points to 51.4 percent.
- 2016 total revenues guidance increased to $22.5-$22.8 billion; EPS guidance increased to $9.55-$9.90 on a GAAP basis and $11.10-$11.40 on a non-GAAP basis.
- The Company generated $2.5 billion of free cash flow.

"We delivered another strong quarter and are on track to meet or exceed our long-term objectives," said Robert A. Bradway, chairman and chief executive officer. "We are in the early stages of a new product launch cycle and have several additional pipeline opportunities rapidly nearing regulatory milestones."

$Millions, except EPS and percentages	Q2'16	Q2'15	YOY Δ
Total Revenues	$ 5,688	$ 5,370	6%
GAAP Operating Income	$ 2,380	$ 2,076	15%
GAAP Net Income	$ 1,870	$ 1,653	13%
GAAP EPS	$ 2.47	$ 2.15	15%
Non-GAAP Operating Income	$ 2,812	$ 2,551	10%
Non-GAAP Net Income	$ 2,146	$ 1,977	9%
Non-GAAP EPS	$ 2.84	$ 2.57	11%

Product Sales Performance

- Total product sales increased 5 percent for the second quarter of 2016 versus the second quarter of 2015. The increase was driven by ENBREL, Prolia, KYPROLIS and XGEVA.
- ENBREL sales increased 10 percent driven by net selling price, offset partially by the impact of competition.
- Neulasta® (pegfilgrastim) sales decreased 1 percent driven by lower unit demand, offset partially by net selling price in the United States (U.S.).
- Aranesp® (darbepoetin alfa) sales increased 5 percent. Unit demand grew due to a shift by some U.S. dialysis customers from EPOGEN® (epoetin alfa) to Aranesp. Unit demand growth was offset partially by unfavorable changes in inventory and net selling price.
- Prolia sales increased 30 percent driven by higher unit demand.
- Sensipar/Mimpara® (cinacalcet) sales increased 13 percent driven by net selling price and higher unit demand.
- XGEVA sales increased 15 percent driven mainly by higher unit demand and, to a lesser extent, net selling price.
- EPOGEN sales decreased 33 percent driven by the impact of competition and, to a lesser extent, a shift by some U.S. dialysis customers to Aranesp.
- NEUPOGEN® (filgrastim) sales decreased 23 percent driven by the impact of competition in the U.S.
- KYPROLIS sales increased 45 percent driven by higher unit demand.
- Vectibix® (panitumumab) sales were flat.
- Nplate® (romiplostim) sales increased 14 percent driven by higher unit demand.
- BLINCYTO® (blinatumomab) sales increased 76 percent driven by higher unit demand.

PRODUCT SALES DETAIL BY PRODUCT AND GEOGRAPHIC REGION

$Millions, except percentages	Q2'16			Q2'15	YOY Δ
	US	ROW	TOTAL	TOTAL	TOTAL
Enbrel®	$1,423	$61	$1,484	$1,348	10%
Neulasta®	962	187	1,149	1,158	(1%)
Aranesp®	260	244	504	479	5%
Prolia®	286	155	441	340	30%
Sensipar® / Mimpara®	303	86	389	344	13%
XGEVA®	275	106	381	331	15%
EPOGEN®	331	0	331	491	(33%)
NEUPOGEN®	141	55	196	256	(23%)
KYPROLIS®	142	30	172	119	45%
Vectibix®	52	108	160	160	0%
Nplate®	84	58	142	125	14%
BLINCYTO®	21	9	30	17	76%
Repatha®	20	7	27	0	*
Other**	17	51	68	57	19%
Total product sales	$4,317	$1,157	$5,474	$5,225	5%

* Not meaningful

** Other includes MN Pharma, Bergamo, IMLYGIC® and Corlanor®

Operating Expense, Operating Margin and Tax Rate Analysis

On a GAAP basis:

- Cost of Sales margin improved by 1.6 percentage points driven primarily by manufacturing efficiencies and higher net selling price. Research & Development (R&D) expenses decreased 7 percent driven primarily by transformation and process improvement efforts and lower spending required to support certain later-stage clinical programs.Selling, General & Administrative (SG&A) expenses increased 11 percent driven primarily by investments in new product launches. Total Operating Expenses were flat year-over-year, with all expense categories reflecting savings from our transformation and process improvement efforts.

- Operating Margin improved by 3.8 percentage points to 43.5 percent.
- Tax Rate decreased by 2.0 percentage points, reflecting discrete benefits associated with tax incentives and the adoption of Accounting Standards Update 2016-09, Improvements to Employee Share-Based Payment Accounting(ASU 2016-09), offset partially by unfavorable changes in the geographic mix of earnings.

On a non-GAAP basis:

- Cost of Sales margin improved by 1.6 percentage points driven primarily by manufacturing efficiencies and higher net selling price. R&D expenses decreased 4 percent driven primarily by transformation and process improvement efforts and lower spending required to support certain later-stage clinical programs. SG&A expenses increased 13 percent driven primarily by investments in new product launches. Total Operating Expenses increased 2 percent, with all expense categories reflecting savings from our transformation and process improvement efforts.
- Operating Margin improved by 2.6 percentage points to 51.4 percent.
- Tax Rate decreased by 1.4 percentage points, reflecting discrete benefits associated with tax incentives and the adoption of ASU 2016-09, offset partially by unfavorable changes in the geographic mix of earnings.

$Millions, except percentages	GAAP			Non-GAAP		
	Q2'16	Q2'15	YOY Δ	Q2'16	Q2'15	YOY Δ
Cost of Sales	$1,050	$1,089	(4%)	$738	$789	(6%)
% of product sales	19.2%	20.8%	(1.6) pts	13.5%	15.1%	(1.6) pts
Research & Development	$900	$964	(7%)	$878	$918	(4%)
% of product sales	16.4%	18.4%	(2.0) pts	16.0%	17.6%	(1.6) pts
Selling, General & Administrative	$1,292	$1,160	11%	$1,260	$1,112	13%
% of product sales	23.6%	22.2%	1.4 pts	23.0%	21.3%	1.7 pts
Other	$66	$81	(19%)	$0	$0	0%
TOTAL Operating Expenses	$3,308	$3,294	0%	$2,876	$2,819	2%
Operating Margin						
operating income as a % of product sales	43.5%	39.7%	3.8 pts	51.4%	48.8%	2.6 pts
Tax Rate	15.2%	17.2%	(2.0) pts	18.6%	20.0%	(1.4) pts

pts: percentage points

Cash Flow and Balance Sheet

- The Company generated $2.5 billion of free cash flow in the second quarter of 2016 versus $3.2 billion in the second quarter of 2015. The decrease was driven by the timing of tax payments and the termination of foreign exchange forward contracts in the second quarter of 2015.
- The Company's third quarter 2016 dividend of $1.00 per share declared on July 22, 2016, will be paid on Sept. 8, 2016, to all stockholders of record as of Aug. 17, 2016.
- During the second quarter, the Company repurchased 3.9 million shares of common stock at a total cost of $591 million. At the end of the second quarter, the Company had $3.6 billion remaining under its stock repurchase authorization.

$Billions, except shares	Q2'16	Q2'15	YOY Δ
Operating Cash Flow	$2.7	$3.3	($0.6)
Capital Expenditures	0.2	0.1	0.1
Free Cash Flow	2.5	3.2	(0.7)
Dividends Paid	0.8	0.6	0.2
Share Repurchase	0.6	0.5	0.1
Avg. Diluted Shares (millions)	756	768	(12)
Cash and Investments	35.0	30.0	5.0

Debt Outstanding	33.2	32.0	1.2
Stockholders' Equity	30.1	27.5	2.6
Note: Numbers may not add due to rounding			

2016 Guidance

For the full year 2016, the Company now expects:

- Total revenues in the range of $22.5 billion to $22.8 billion.
  - Previously, the Company expected total revenues in the range of $22.2 billion to $22.6 billion.
- On a GAAP basis, EPS in the range of $9.55 to $9.90 and a tax rate in the range of 16.5 percent to 17.5 percent.
  - Previously, the Company expected GAAP EPS in the range of $9.34 to $9.74. Tax rate guidance is unchanged.
- On a non-GAAP basis, EPS in the range of $11.10 to $11.40 and a tax rate in the range of 19.0 percent to 20.0 percent.
  - Previously, the Company expected non-GAAP EPS in the range of $10.85 to $11.20. Tax rate guidance is unchanged.
- Capital expenditures to be approximately $700 million.

SECOND QUARTER PRODUCT AND PIPELINE UPDATE
Key development milestones:

Clinical Program	Indication	Milestone
Repatha® (evolocumab)	Hyperlipidemia	Phase 3 coronary imaging data expected H2 2016 Phase 3 CV outcomes data expected Q1 2017*
KYPROLIS	Newly diagnosed multiple myeloma	Phase 3 data expected H2 2016*
BLINCYTO®	Pediatric Ph- R/R B-cell precursor ALL	FDA priority review
Parsabiv™ (etelcalcetide)†	Secondary hyperparathyroidism	Global regulatory reviews
XGEVA	Prevention of SREs in multiple myeloma	Phase 3 data expected H2 2016*
Romosozumab	Postmenopausal osteoporosis	US regulatory review Global regulatory submissions
Erenumab (AMG 334)	Migraine Prophylaxis	Phase 3 episodic migraine data expected H2 2016
ABP 215 (biosimilar bevacizumab)	Oncology	Global regulatory submissions
ABP 501 (biosimilar adalimumab)	Inflammatory diseases	Global regulatory reviews
ABP 980 (biosimilar trastuzumab)	Breast Cancer	Global regulatory submissions

The Company provided the following updates on selected product and pipeline programs:

Repatha

- In July, the U.S. Food and Drug Administration (FDA) approved the Repatha *Pushtronex*™ system (on-body infusor with prefilled cartridge) for monthly single-dose administration.
- Data from a Phase 3 study evaluating the effects of Repatha on atherosclerotic disease as measured by intravascular ultrasound are expected in H2 2016.
- Data from an event driven Phase 3 study evaluating the effects of Repatha on cardiovascular outcomes are expected in Q1 2017.

KYPROLIS

- In June, the European Commission approved an expanded indication for KYPROLIS, to be used in combination with dexamethasone alone, for adult patients with multiple myeloma who have received at least one prior therapy, based on the ENDEAVOR data.

- Data from the event driven Phase 3 CLARION study of KYPROLIS versus bortezomib in newly diagnosed, transplant ineligible multiple myeloma patients is expected in H2 2016.

BLINCYTO

- In May, FDA accepted for priority review the supplemental Biologics License Application for BLINCYTO to include new data supporting the treatment of pediatric and adolescent patients with Philadelphia chromosome-negative relapsed or refractory B-cell precursor acute lymphoblastic leukemia. The Prescription Drug User Fee Act target action date is Sept. 1, 2016.

Romosozumab

- In July, a Biologics License Application for romosozumab for the treatment of osteoporosis in postmenopausal women at increased risk for fracture was submitted to FDA.

Erenumab

- In June, a global Phase 2 study evaluating the efficacy and safety of erenumab in chronic migraine prevention met its primary endpoint.

ABP 980

- In July, the primary analysis was completed for a Phase 3 study evaluating the efficacy and safety of ABP 980 compared with trastuzumab in patients with human epidermal growth factor receptor 2-positive early breast cancer.

http://www.amgen.com/media/news-releases/2016/07/amgen-reports-second-quarter-2016-financial-results/

BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) - BioMarin Provides Positive Proof-of-Concept Data for BMN 270 Gene Therapy in Hemophilia A in Late Breaking Oral Presentation at the World Federation of Hemophilia (WFH) 2016 World Congress – 27/7/2016

6 of 7 High Dose Patients Show Factor VIII levels above 50%, 7th patient above 10%

No Clinically Relevant Sustained Rises in ALT

Phase 2b Study to Begin Mid-2017 for Potential Accelerated Approval Filing

Conference Call and Web-cast with Slides to be Held Wednesday, July 27th at 4:05pm ET

SAN RAFAEL, Calif., July 27, 2016 (GLOBE NEWSWIRE) - BioMarin Pharmaceutical Inc. (NASDAQ:BMRN) announced today positive interim results of an open-label Phase 1/2 study of BMN 270, an investigational gene therapy treatment for severe hemophilia A at the XXXII International Congress of the World Federation of Hemophilia (WFH). The data was presented in the Late Breaking Gene Therapy session by John Pasi, Professor of Haemostasis and Thrombosis, Barts and the London School of Medicine, Honorary Consultant Haematologist, The Royal London Hospital, and a lead investigator of the study. The data presented at the congress is an update since the Company reported initial results on this same study on April 20, 2016. To access the data presented at the Congress, click here.

A total of nine patients with severe hemophilia A received a single dose of BMN 270, seven of whom have been treated at the highest dose of 6 x 1013 vg/kg. As of the July 6 data cut off, post-treatment follow-up ranges from 12 to 28 weeks. For the seven patients treated with the high dose, as of each patients' most recent reading, six of seven patients had Factor VIII levels above 50%, as a percentage calculated based on the numbers of International Units per deciliter of plasma (IU/dL), and the seventh was above 10%. In addition, four patients who have been followed the longest had a mean Factor VIII level of 146% at their 20 week visit. Two patients with Factor VIII levels above 200% had no unexpected events or need for medical intervention. For the seven patients at the high dose, the median annualized bleeding rate measured from day of gene transfer to data cut of observation period fell to 5 from 20.

No clinically relevant sustained rises in ALT levels or other markers of liver toxicity have been observed. The maximum ALT levels were between 23 and 82 U/L (less than two times the upper limit of normal, which is 43 U/L for

the central laboratory in this study) approximately 12 weeks after gene delivery and generally declined over the next few weeks. ALT rises have not been associated with any decrease in Factor VIII levels. A steroid regimen administered to all high dose patients has been well-tolerated. Patients are successfully tapering off of steroids with two subjects off steroid therapy for up to 2.5 weeks with no adverse impact on Factor VIII expression or ALT levels. Study medication was generally well tolerated. No serious adverse events were observed, and most common adverse events were mild in severity.

"These data provide strong proof of concept evidence that restoration of clotting function may be achieved by gene therapy," said John Pasi, Ph.D. F.R.C.P, Professor of Haemostasis and Thrombosis at Barts and the London School of Medicine and Dentistry and primary investigator for the BMN 270 Phase 1/2 clinical trial. "For the first time, patients have reason to hope to avoid bleeding and the opportunity to live a normal life."

"We look forward to collaborating with experts and health authorities to design the next phase of investigation," said Hank Fuchs, M.D., Chief Medical Officer at BioMarin. "Beginning in mid-2017, a Phase 2b study will seek to evaluate the optimal dose of BMN 270 using Factor VIII expression as the primary endpoint with material from the to-be-commercialized manufacturing process. If successful, this study could support an accelerated approval given the severe unmet need, the substantial effect and tolerability of the treatment."

Phase 1/2 Study Design

The current Phase 1/2 study is evaluating the safety and efficacy of BMN 270 gene therapy in up to 12 patients with severe hemophilia A, as defined by the WFH as less than 1% of blood clotting factor. The primary endpoints are to assess the safety of a single intravenous administration of a recombinant AAV vector coding for human-coagulation factor VIII and to determine the change from baseline of factor VIII expression level at 16 weeks after infusion. The kinetics, duration and magnitude of AAV-mediated factor VIII activity in individuals with hemophilia A will be determined and correlated to an appropriate BMN 270 dose.

This is a dose escalation study with the goal of observing an increase in factor VIII levels. Secondary endpoints include assessing the impact of BMN 270 on the frequency of factor VIII replacement therapy, the number of bleeding episodes requiring treatment and any potential immune responses. Patients will be monitored for safety and durability of effect for five years.

About Hemophilia A

Hemophilia A, also called factor VIII (FVIII) deficiency or classic hemophilia, is a genetic disorder caused by missing or defective factor VIII, a clotting protein. Although it is passed down from parents to children, about 1/3 of cases are caused by a spontaneous mutation, a new mutation that was not inherited.1 As an X-linked disorder, hemophilia A mostly affects males, occurring in approximately 1 in 5,000 male births.2 People living with the disease are not able to form blood clots efficiently and are at risk for excessive bleeding from modest injuries, potentially endangering their life. People with severe hemophilia often bleed spontaneously into their muscles or joints. The standard of care for the 43% of hemophilia A patients who are severely affected, is a prophylactic regimen of factor VIII infusions three times per week.3 Even with prophylactic regimens, many patients still experience microbleeds and spontaneous bleeding events that result in progressive joint damage.

About Gene Therapy

Gene therapy is a treatment designed to alter a genetic problem by adding a corrected copy of the defective gene. The functional gene is inserted into a vector — containing a DNA sequence coding for a specific protein — that acts as a delivery mechanism, providing the ability to deliver the functional gene to cells. The cells can then use the information to build the functional protein that the body needs, potentially reducing or eliminating the cause of the disease. Currently, gene therapy for the treatment of hemophilia A is available only as part of a clinical trial. The AAV approach to gene therapy has been advanced at the University College London (UCL) in the treatment of Hemophilia B. At UCL, this technology has shown evidence to be both safe and effective, correcting bleeding for greater than four years in a continuing clinical trial.

http://investors.bmrn.com/releasedetail.cfm?ReleaseID=981465

BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) - FDA Accepts BLA for BioMarin's Cerliponase Alfa for CLN2 Disease, Form of Batten Disease – 27/7/2016

Potential First Treatment for Fatal, Rare, Brain Disease in Children

FDA Grants Priority Review Status and Breakthrough Therapy Designation

PDUFA Action Date is January 27, 2017

MAA Submitted to European Regulatory Authorities

SAN RAFAEL, Calif., July 27, 2016 (GLOBE NEWSWIRE) - BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that the U.S. Food and Drug Administration (FDA) accepted for review the submission of a Biologics License Application (BLA) for cerliponase alfa, an investigational therapy to treat children with CLN2 disease, a form of Batten disease. The Prescription Drug User Fee Act (PDUFA) goal date for a decision is January 27, 2017. BioMarin also has submitted a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for cerliponase alfa, and it is undergoing validation at the Agency.

The FDA granted cerliponase alfa Priority Review status, which is designated to drugs that offer major advances in treatment or provide a treatment where no adequate therapy exists. Cerliponase alfa was previously granted Orphan Drug Designation and Breakthrough Therapy Designation by the FDA.

The company also announced that the preliminary approved brand name for cerliponase alfa is Brineura™.

"CLN2 disease is a rapidly progressing, fatal neurodegenerative disease with no approved treatments. The FDA recognized the potential of cerliponase alfa to help address this devastating condition, and we look forward to working closely with the Agency over the coming months," said Hank Fuchs, M.D., Chief Medical Officer of BioMarin. "We thank the community for its continued support, as well as the patients and families who dedicated their time to the clinical development of cerliponase alfa."

"This is an historic milestone for the Batten disease community. For the first time since it was originally described more than a century ago, there is a potential treatment for our children with CLN2 disease," said Margie Frazier, PhD, Executive Director of the Batten Disease Support and Research Association. "We appreciate BioMarin's continued commitment to pursue a therapy for this devastating disease and to advancing it quickly through the development process."

Children with CLN2 disease typically begin to present symptoms between the ages of two and four, with the majority of affected children losing their ability to walk and talk by approximately six years of age. Initial symptoms can include language delay and seizures, followed by movement disorders, motor deterioration, dementia and blindness. During the later stages of the disease, feeding and tending to everyday needs become very difficult, and death often occurs between 8 and 12 years of age.

Early Access Program

BioMarin is planning to implement an early access program to provide access to treatment for additional CLN2 patients prior to obtaining marketing approval. The program will be limited in scope and number of participants and will be conducted under a protocol. BioMarin expects that the program will be conducted initially at centers that have participated in the cerliponase alfa study. Those sites have experience administering this drug directly to the brain and would ensure continued patient monitoring.

The timing of the start of the program is on track, and the initial site is expected to be open during Q3 2016. The exact timing will vary by country of the sites participating. The overall scope and details of this program are still being determined. BioMarin must adhere to specific legal procedures for each country and has begun these preparations with the goal of being ready to dose patients in Q3 2016.

About Cerliponase Alfa

Cerliponase alfa is a recombinant form of human tripeptidyl peptidase 1 (TPP1), the enzyme deficient in patients with CLN2 disease. It is an enzyme replacement therapy designed to restore TPP1 enzyme activity and break down the storage materials that cause CLN2 disease. In order to reach the cells of the brain and central nervous system, the treatment is delivered directly to the fluid surrounding the brain (cerebrospinal fluid) by intracerebroventricular (ICV) infusion, an established technique for delivering drugs to the brain.

For additional information regarding the investigational product cerliponase alfa, please contact BioMarin Medical Information at medinfo@bmrn.com.

About CLN2 Disease

CLN2 disease is caused by mutations in the TPP1/CLN2 gene, resulting in deficient activity of the enzyme TPP1. In the absence of TPP1, lysosomal storage materials normally metabolized by this enzyme accumulate in many organs, particularly in the brain and retina. Buildup of these storage materials in the cells of the nervous system contribute to progressive and relentless neurodegeneration, which manifests as loss of cognitive, motor and visual functions.

There is no approved treatment that can prevent, stop or reverse CLN2 disease. Symptomatic care to treat disease symptoms, prevent and treat complications, and attempt to preserve quality of life is the only currently available option for patients with this rare disease.

http://investors.bmrn.com/releasedetail.cfm?ReleaseID=981569

Vertex Pharmaceuticals (NASDAQ: VRTX) - Vertex Reports Second Quarter 2016 Financial Results – 27/7/2016

-Second quarter 2016 cystic fibrosis product revenues of $426 million; $245 million for ORKAMBI® (lumacaftor/ivacaftor) and $180 million for KALYDECO® (ivacaftor)-

-Vertex reiterates 2016 guidance for ORKAMBI product revenues of $1.0 to $1.1 billion and KALYDECO product revenues of $685 to $705 million-

-Pipeline of investigational CF medicines continues to progress and expand with addition of recent Moderna mRNA collaboration-

BOSTON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the quarter ended June 30, 2016 and reviewed recent progress with its approved and investigational cystic fibrosis (CF) medicines. Vertex also reiterated its financial guidance for total 2016 ORKAMBI® and KALYDECO® revenues and expenses. Key financial results include:

	Three Months Ended June 30,		
	2016	2015	% Change
	(in millions, except per share and percentage data)		
ORKAMBI product revenues, net	$ 245	$ —	N/A

KALYDECO product revenues, net	$ 180	$ 155	16%
TOTAL CF product revenues, net	$ 426	$ 155	175%
GAAP net loss	$ (65)	$ (189)	(66)%
GAAP net loss per share	$ (0.26)	$ (0.78)	(67)%
Non-GAAP net income (loss)	$ 58	$ (131)	N/A
Non-GAAP net income (loss) per share	$ 0.24	$ (0.54)	N/A

"Just over a year ago, we received FDA approval for ORKAMBI, marking the most significant step to date in our journey to develop new medicines for potentially all people with CF," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "Today, approximately 27,000 people are eligible for a medicine to treat the cause of their CF, and we're making significant progress toward bringing ORKAMBI and KALYDECO to even more patients while also advancing our pipeline of other potential medicines to enhance the future treatment of CF."

Vertex today reviewed recent progress from across its CF program:

ORKAMBI

Supplemental New Drug Application for the treatment of children ages 6 to 11 accepted for Priority Review by the U.S. FDA: In late May 2016, the U.S. Food and Drug Administration (FDA) granted Vertex's request for Priority Review of a supplemental New Drug Application (sNDA) for approval of ORKAMBI for children ages 6 through 11 who have two copies of the F508del mutation. The FDA set a target review date of September 30, 2016 for a decision on the sNDA. There are approximately 2,400 children ages 6 through 11 who have two copies of the F508del mutation in the U.S.The sNDA was based on data from an open label Phase 3 safety study of ORKAMBI. Data from this study were presented at the 39th European Cystic Fibrosis Society (ECFS) conference on June 10, 2016.

Enrollment complete in Phase 3 study in children ages 6 to 11 to support approval in Europe: Vertex has completed enrollment in a six-month Phase 3 efficacy study evaluating ORKAMBI in children ages 6 through 11 who have two copies of the F508del mutation. The primary endpoint is the absolute change in lung clearance index. Pending data from the study, Vertex plans to submit a Marketing Authorization Application variation in theEuropean Union in the first half of 2017. In Europe, there are approximately 3,400 children ages 6 through 11 who have two copies of the F508del mutation.

Initiation of Phase 3 study of ORKAMBI in children ages 2 to 5: Vertex recently initiated a Phase 3 study of ORKAMBI in children ages 2 to 5. Similar to the study of KALYDECO in children in this age group, the first part of the two-part study is evaluating pharmacokinetics and safety to inform dose selection for the second part of the study. The primary endpoint of the second part of the study is safety and tolerability, with multiple efficacy measurements as secondary endpoints.

KALYDECO

Regulatory filing for patients with residual function mutations: In October 2015, Vertex submitted an sNDA for approval of KALYDECO for treatment of people with CF ages 2 and older who have one of 23 residual function mutations and received a Complete Response Letter on this sNDA inFebruary 2016. There are approximately 1,500

people ages 2 and older in the U.S. who have one of the 23 residual function mutations included in the sNDA, and Vertex continues to pursue FDA approval of KALYDECO for these patients as soon as possible.

VX-661 in Combination with Ivacaftor

Data from Phase 3 study in people with two copies of F508del mutation expected in first half of 2017: Vertex today announced that it expects to complete enrollment of a 24-week Phase 3 placebo-controlled study evaluating the investigational combination of VX-661 and ivacaftor in people ages 12 and older who have two copies of the F508del mutation in August 2016. Data from this study are expected in the first half of 2017. The remaining three Phase 3 studies of VX-661 in combination with ivacaftor are proceeding as outlined in the company's April 27, 2016 press release. Vertex plans to submit a New Drug Application (NDA) to the FDA for VX-661 in combination with ivacaftor in the second half of 2017, pending data from the Phase 3 program.

Next-Generation Correctors

Ongoing Phase 1 studies in healthy volunteers: Vertex's two next-generation correctors known as VX-152 and VX-440 are being evaluated alone and as part of a triple combination with VX-661 and ivacaftor in ongoing Phase 1 studies in healthy volunteers. Pending data from the Phase 1 studies, the company expects to begin Phase 2 clinical development in people with CF to evaluate one or both of the next-generation correctors with VX-661 and ivacaftor in the second half of 2016.

New Collaboration to Advance Future Treatment of CF

Collaboration with Moderna Therapeutics focused on mRNA Therapeutics for CF: In early July, Vertex entered into an exclusive research collaboration and licensing agreement with Moderna Therapeutics aimed at the discovery and development of messenger Ribosomal Nucleic Acid (mRNA) therapies for the treatment of CF. The collaboration will focus on the use of mRNA therapies to treat the underlying cause of CF by enabling cells in the lungs to produce functional copies of the cystic fibrosis transmembrane conductance regulator (CFTR) protein, which is known to be defective in people with CF. As part of the collaboration, Vertex made an up-front payment of $20 million to Moderna as well as a $20 million equity investment. The investment will provide Vertex with an ownership stake in Moderna. Vertex will also pay Moderna future development and regulatory milestones of up to$275 million, including $220 million in approval and reimbursement milestones, as well as tiered royalty payments on future sales.
Second Quarter 2016 Financial Highlights

Revenues:

- Net product revenues from ORKAMBI were $245.5 million. ORKAMBI was launched in the U.S. in July 2015.
- Net product revenues from KALYDECO were $180.2 million, compared to $154.9 million for the second quarter of 2015.

Expenses:

- GAAP operating expenses were $428.3 million compared to $337.2 million for the second quarter of 2015. Non-GAAP operating expenses (combined non-GAAP R&D and SG&A) were $306.3 million compared to $253.9 million for the second quarter of 2015. The increases were primarily driven by increased costs related to the progression of our CF pipeline and to increased investment in global commercial support for the launch of ORKAMBI.
- GAAP R&D expenses were $271.0 million compared to $223.9 million for the second quarter of 2015. Non-GAAP R&D expenses were $217.7 millioncompared to $181.9 million for the second quarter of 2015. The increases were primarily driven by increased investment to progress our portfolio of CF medicines.

- GAAP SG&A expenses were $111.7 million compared to $94.4 million for the second quarter of 2015. Non-GAAP SG&A expenses were $88.6 millioncompared to $72.0 million for the second quarter of 2015. The increases were primarily driven by increased investment to support the global launch of ORKAMBI.

Net Income (Loss) Attributable to Vertex:

- GAAP net loss was $(64.5) million, or $(0.26) per diluted share, compared to GAAP net loss of $(188.8) million, or $(0.78) per diluted share, for the second quarter of 2015. Non-GAAP net income was $58.0 million, or $0.24 per diluted share, compared to a non-GAAP net loss of $(130.7) million, or$(0.54) per diluted share, for the second quarter of 2015.

Cash Position:

- As of June 30, 2016, Vertex had $1.07 billion in cash, cash equivalents and marketable securities compared to $1.04 billion in cash, cash equivalents and marketable securities as of December 31, 2015.
- As of June 30, 2016, Vertex had $300 million outstanding from a credit agreement, repayable by the end of the third quarter of 2017.

2016 Financial Guidance:

Vertex today reiterated its 2016 revenue guidance for ORKAMBI and KALYDECO. The company also reiterated guidance for its 2016 combined non-GAAP R&D and SG&A expenses. The guidance is summarized below:

- ORKAMBI: The company continues to expect total 2016 product revenues for ORKAMBI of $1.0 to $1.1 billion. As of June 30, 2016, approximately 6,000 patients had initiated treatment with ORKAMBI in the U.S. In addition to revenues from the use of ORKAMBI in patients ages 12 and older in theU.S., the 2016 ORKAMBI guidance also reflects potential revenues from the anticipated use of ORKAMBI in the U.S. for the treatment of people ages 6 to 11 who have two copies of the F508del mutation in the fourth quarter of 2016, pending FDA approval, and revenues from sales of ORKAMBI outside the U.S., primarily in Germany.
- KALYDECO: The company continues to expect total 2016 product revenues for KALYDECO of $685 to $705 million. 2016 guidance for KALYDECO currently excludes any revenues related to the potential approval of KALYDECO for people in the U.S. who have residual function mutations.
- Operating Expenses (Combined Non-GAAP R&D and SG&A Expenses): Vertex continues to expect that its combined non-GAAP R&D and SG&A expenses in 2016 will be in the range of $1.18 to $1.23 billion. Vertex's expected non-GAAP R&D and SG&A expenses exclude stock-based compensation expense and certain other expenses.

http://investors.vrtx.com/releasedetail.cfm?ReleaseID=981478

Seattle Genetics (NASDAQ: SGEN) - Seattle Genetics Reports Second Quarter 2016 Financial Results – 26/7/2016

-Second Quarter 2016 Revenues Were $95.4 Million, Including Record $66.2 Million in ADCETRIS® (Brentuximab Vedotin) U.S. and Canada Net Sales-

-Top-Line Data from ADCETRIS Phase 3 ALCANZA Trial Expected in Third Quarter of 2016-

-Conference Call Today at 4:30 p.m. ET-

BOTHELL, Wash. - (BUSINESS WIRE) - Jul. 26, 2016 - Seattle Genetics, Inc. (NASDAQ: SGEN) today reported financial results for the second quarter ended June 30, 2016. The company also highlighted ADCETRIS (brentuximab

vedotin) commercialization and clinical development accomplishments, vadastuximab talirine (SGN-CD33A; 33A) activities and progress with its pipeline of antibody-drug conjugates (ADCs) and other proprietary programs.

“We reported record ADCETRIS net sales in the second quarter, which were up 20 percent for the quarter and year-to-date compared to the same periods in 2015. To expand on the ADCETRIS opportunity, we are executing on three ongoing phase 3 clinical trials that are approaching data, starting with ALCANZA top-line results this quarter,” said Clay Siegall, Ph.D., President and Chief Executive Officer of Seattle Genetics. “We also demonstrated progress in the second quarter with our clinical-stage pipeline towards our goal of becoming a multi-product oncology company. We advanced 33A into a phase 3 trial for acute myeloid leukemia (AML) and reported encouraging phase 1 data from two ADCs for urothelial cancer, ASG-15ME and enfortumab vedotin (ASG-22ME). We anticipate advancing several new programs and generating additional data from our pipeline over the remainder of 2016.”

Recent ADCETRIS Highlights

The European Commission approved ADCETRIS for the treatment of adult patients with CD30+ Hodgkin lymphoma at increased risk of relapse or progression following autologous stem cell transplant based on data from the phase 3 AETHERA clinical trial. This is the third approved indication for ADCETRIS in the European Union.

Announced that final data from the ADCETRIS monotherapy pivotal phase 2 clinical trial in relapsed or refractory classical Hodgkin lymphoma were published in the journal Blood. The manuscript, which summarizes the five-year, end-of-study results, highlights that many patients who achieved a complete remission remained in remission at the time of this final analysis.

Takeda continues to receive additional marketing approvals for ADCETRIS, which is now commercially available in 65 countries worldwide.

Recent Vadastuximab Talirine (SGN-CD33A) Highlights

Initiated the pivotal phase 3 CASCADE clinical trial evaluating 33A in combination with the hypomethylating agents (HMAs) azacitidine or decitabine in approximately 500 older patients with newly diagnosed AML. The trial is designed to determine if the 33A-containing regimen improves overall survival compared to patients receiving HMAs alone.

Reported data from a phase 1 trial of 33A plus HMAs in older, newly diagnosed patients with AML in an oral presentation at the 21st Congress of the European Hematology Association (EHA). The data showed a 76 percent objective response rate, including a 41 percent complete remission rate with manageable tolerability profile. The median overall survival for all patients in the phase 1 trial is interim and expected to evolve. The estimated median overall survival for the first 25 patients enrolled in the study was 12.75 months.

Recent Pipeline and Other Highlights

Reported data at the American Society of Clinical Oncology (ASCO) annual meeting from phase 1 trials of ASG-15ME and enfortumab vedotin in metastatic urothelial cancer, primarily bladder carcinoma. The data showed that both ADCs had manageable safety profiles and objective response rates of 40 to 50 percent at the likely recommended doses for future development. ASG-15ME and enfortumab vedotin are being co-developed with Astellas.

Triggered milestones under ongoing ADC collaborations based on progress with programs utilizing Seattle Genetics technology, including from:

Astellas, upon its initiation of a phase 2 clinical trial in metastatic renal cell carcinoma; and,

AbbVie, based on progress with a preclinical program.

Added to and promoted several members of the senior management team, including:

Promoting Naomi Hunder, M.D., to Vice President, Clinical Development. Dr. Hunder joined Seattle Genetics in 2010. She has most recently served as the clinical lead for the ADCETRIS program, notably for the company's successful FDA approval in post-autologous transplant high-risk Hodgkin lymphoma based on data from the phase 3 AETHERA trial.

Promoting Dana Kennedy, Pharm.D., to Vice President, Clinical Development. Dr. Kennedy joined Seattle Genetics in 2007. Her contributions have included the clinical development work that led to the approval of ADCETRIS in systemic anaplastic large cell lymphoma and serving as clinical and program leader for SGN-CD33A.

Hiring Ian Pyrah, Ph.D., as Vice President, Non-Clinical Sciences. Prior to joining Seattle Genetics, Dr. Pyrah spent 10 years at Amgen in several leadership roles, including responsibility for the non-clinical component of a number of successful regulatory submissions.

Hiring Venkat Ramanan, Ph.D., as Vice President, Finance. Dr. Ramanan previously spent nine years at Gilead Sciences and prior to that he was at Amgen and ZS Associates. He has served in a range of roles in finance, business planning and operations supporting U.S. and international markets.

Anticipated ADCETRIS Upcoming Activities

Report top-line data in the third quarter of 2016 from the phase 3 ALCANZA trial in patients with CD30-expressing cutaneous T-cell lymphoma (CTCL).

Report data in the 2017 through mid-2018 timeframe from the phase 3 ECHELON-1 trial in frontline classical Hodgkin lymphoma.

Complete enrollment in the phase 3 ECHELON-2 trial in frontline mature T-cell lymphoma (MTCL) during 2016 and report data in the 2017 to 2018 timeframe.

ADCETRIS is not currently approved for use in CTCL, frontline Hodgkin lymphoma or frontline MTCL.

Anticipated Vadastuximab Talirine (SGN-CD33A) Upcoming Activities

Continue clinical site initiations and enrollment of 500 patients to the pivotal phase 3 CASCADE clinical trial evaluating 33A in combination with HMAs in older patients with newly diagnosed AML.

Report data from ongoing phase 1 trials, including a phase 1b trial of 33A in combination with cytarabine and daunorubicin (7+3) for frontline, younger AML patients.

More information about 33A and ongoing clinical trials can be found at www.ADC-CD33.com.

Anticipated Pipeline Programs Upcoming Activities

Initiate a randomized phase 2 trial of denintuzumab mafodotin (SGN-CD19A; 19A) in frontline diffuse large B-cell lymphoma (DLBCL) during 2016.

Report additional data from phase 1 trials of ASG-15ME and enfortumab vedotin at the European Society for Medical Oncology (ESMO) annual congress being held October 7 to 11, 2016 in Copenhagen, Denmark.

Report clinical data during 2016 from other pipeline programs, including SGN-LIV1A.

Initiate a phase 1 trial of SGN-CD123A in relapsed or refractory AML. SGN-CD123A is an ADC targeted to CD123 utilizing Seattle Genetics' newest technology, comprising an engineered cysteine antibody (EC-mAb) stably linked to a highly potent DNA binding agent called a pyrrolobenzodiazepine (PBD) dimer. CD123 is expressed across AML subtypes, and is particularly prominent on leukemic stem cells.

Advance SGN-CD352A, a novel ADC for multiple myeloma, into a phase 1 clinical trial. SGN-CD352A targets CD352, and utilizes the company's PBD and EC-mAb technology. CD352 is highly expressed on multiple myeloma as well as B-cell malignancies, including chronic lymphocytic leukemia and non-Hodgkin lymphoma.

Second Quarter and Six Months 2016 Financial Results

Total revenues in the quarter and six month periods ended June 30, 2016 increased to $95.4 million and $206.6 million, respectively, compared to $77.1 million and $159.3 million from the same periods in 2015. Revenues included:

ADCETRIS net sales in the second quarter were $66.2 million, a 20 percent increase from net sales of $55.1 million in the second quarter of 2015. For the year-to-date, ADCETRIS sales were $124.9 million, compared to $104.0 million for the year-to-date period in 2015, a 20 percent increase.

Royalty revenues in the second quarter of 2016 were $9.2 million, compared to $7.6 million in the second quarter of 2015. For the year-to-date in 2016, royalty revenues were $41.5 million, compared to $18.7 million for the first six months of 2015. Royalty revenues are primarily driven by international sales of ADCETRIS by Takeda. Royalty revenues for the year-to-date in 2016 also included a $20.0 million sales milestone payment from Takeda earned in the first quarter of 2016.

Amounts earned under the company's ADCETRIS and ADC collaborations totaled $20.0 million in the second quarter and $40.2 million for the first six months of 2016, compared to $14.4 million and $36.6 million for the same periods in 2015.

Total costs and expenses for the second quarter of 2016 were $128.8 million, compared to $124.7 million for the second quarter of 2015. For the first six months of 2016, total costs and expenses were $261.0 million, compared to $228.6 million in the first six months of 2015. The increase in 2016 costs and expenses was primarily driven by progress with ADCETRIS, 33A clinical development and manufacturing activities and investment in the company's pipeline programs.

Non-cash, share-based compensation cost for the first six months of 2016 was $24.3 million, compared to $17.6 million for the same period in 2015.

Net loss for the second quarter of 2016 was $32.7 million, or $0.23 per share, compared to a net loss of $47.5 million, or $0.38 per share, for the second quarter of 2015. For the six months ended June 30, 2016, net loss was $53.2 million, or $0.38 per share, compared to a net loss of $69.2 million, or $0.55 per share, for the same period in 2015.

As of June 30, 2016, Seattle Genetics had $659.5 million in cash, cash equivalents and investments, compared to $712.7 million as of December 31, 2015.

http://investor.seattlegenetics.com/phoenix.zhtml?c=124860&p=irol-newsArticle&ID=2188443

Latest Research

Industry-academic partnerships: an approach to accelerate innovation

Jennwood Chen, Timothy Pickett, Ashley Langell, Ashley Trane, Brian Charlesworth, Kris Loken, Sarah Lombardo, John T. Langell

Abstract

Background

Biotechnology companies are process-driven organizations and often struggle with their ability to innovate. Universities, on the other hand, thrive on discovery and variation as a source of innovation. As such, properly structured academic-industry partnerships in medical technology development may enhance and accelerate innovation. Through joint industry-academic efforts, our objective was to develop a technology aimed at global cervical cancer prevention.

Methods

Our Center for Medical Innovation assembled a multidisciplinary team of students, surgical residents, and clinical faculty to enter in the University of Utah's annual Bench-to-Bedside competition. Bench-to-Bedside is a university program centered on medical innovation. Teams are given access to university resources and are provided $500.00 for prototype development. Participation by team members are on a volunteer basis. Our industry partner presented the validated need and business mentorship. The team studied the therapeutic landscape, environmental constraints, and used simulation to understand human factors design and usage requirements. A physical device was manufactured by first creating a digital image (SOLIDWORKS 3D CAD). Then, using a 3-dimensional printer (Stratasys Objet30 Prime 3D printer), the image was translated into a physical object. Tissue burn depth analysis was performed on raw chicken breasts warmed to room temperature. Varying combinations of time and temperature were tested, and burn depth and diameter were measured 30 min after each trial. An arithmetic mean was calculated for each corresponding time and temperature combination. User comprehension of operation and sterilization was tested via a participant validation study. Clinical obstetricians and gynecologists were given explicit instructions on usage details and then asked to operate the device. Participant behaviors and questions were recorded.

Results

Our efforts resulted in a functional battery-powered hand-held thermocoagulation prototype in just 72 d. Total cost of development was <$500. Proof of concept trials at 100°C demonstrated an average ablated depth and diameter of 4.7 mm and 23.3 mm, respectively, corresponding to treatment efficacy of all grades of precancerous cervical lesions. User comprehension studies showed variable understanding with respect to operation and sterilization instructions.

Conclusions

Our experience with using industry-academic partnerships as a means to create medical technologies resulted in the rapid production of a low-cost device that could potentially serve as an integral piece of the “screen-and-treat” approach to premalignant cervical lesions as outlined by World Health Organization. This case study highlights the impact of accelerating medical advances through industry-academic partnership that leverages their combined resources.

http://www.sciencedirect.com/science/article/pii/S0022480416301500

The Industry

Industry Trends

Bioscience contributes 1.6 million jobs to the U.S. economy and makes up 60,000 establishments with $120 billion in wages. Within the industry, employment in non-medical testing labs (which is a component of the larger architectural and engineering services industry) is expected to grow 2.3% annually between 2006 and 2016. This is followed by medical and diagnostic labs (2.2%), and scientific research and development (0.9%). On the manufacturing side, employment in pharmaceutical and medicine manufacturing is expected to increase 2.2% annually during the same period, and medical equipment and supplies manufacturing is expected to increase 0.1% annually. Indeed, within manufacturing the biosciences are one of the few bright spots in a sector that is expected to shed over 1.5 million jobs nationwide between 2006 and 2016.

Numerous factors are influencing the growth of the biosciences sector both in the U.S. and abroad. These include a rapidly aging population base in industrialized nations who will require growing levels of healthcare, aggressive investment in biosciences research from both the public and private sectors, growing dependence on genetically-engineered agricultural products, homeland security efforts to develop and stockpile vaccines to combat bioterrorism attacks, growing emphasis on the development of biofuels as a substitute for petroleum, and the continued evolution of faster and more sophisticated information technologies to aid in biosciences research and development.

http://www.maricopa.edu/work/pdfs/SummaryBioscience.pdf

20 Most Promising Biotech Technology Solution Providers

The United States is the largest market and leading consumer of biotechnology products in the world, and home to more than 1,300 firms involved in the industry. The country maintains a competitive environment for the development and commercialization of biotechnology with wideranging, multi-disciplinary activities including recombinant DNA techniques and cloning. Through the world's largest scientific research base and longstanding government support for biomedical and other biotechnology research and development, U.S. is set to race ahead.

In this competitive landscape, companies are surging in designing data management systems in the biotech and pharmaceutical industries, providing a platform that is transforming the healthcare experience by personalizing the process of connecting individuals with health measurement opportunities. The introduction of cloud computing is empowering the industry to transform healthcare through nextgeneration semantic technology, contributing to the evolution of this sector. In research and development, big data and analytics are being adopted across pharmaceutical that has empowered firms across the world to take better decisions and develop eminent insights. Further, with the increasing use of sensors on the manufacturing floor, the network of Internet of Things has the amenities to improve patient outcomes, which will eventually take the biotech vertical to new horizons and beyond.

In the last few months, we have looked at hundreds of solution providers who primarily focus on providing the right expertise and technology to the biotech industry and shortlisted the ones that are in the forefront of tackling key challenges in the industry.

In our selection, we looked at the vendors' capability to fulfill the business objectives through innovative products and services. Also, we evaluated the vendors' support through the integration of latest technologies into their system. We present to you CIOReview's 20 Most PromisingBiotech Technology Solution Providers.

http://www.pepgel.com/PDF/CIO-Review_Pepgel_hig-res.pdf

The Biotechnology Industry Organization (BIO) released a first-of-its-kind study on venture financing broken down by disease area and novelty of research over a ten-year period from 2004 to 2013.

"Since venture financing is the lifeblood of our industry, we wanted to better understand trends in venture financing over the last decade by conducting the broadest, most comprehensive study possible," said Cartier Esham, PhD,

Executive Vice President of Emerging Companies. “The aim was to identify funding trends for emerging drug developers within specific therapeutic areas and across varying levels of innovation.”

The report analyzes data from four venture capital databases – Thomson Reuters, BioCentury, Elsevier, and Evaluate Pharm – to investigate investor trends, examine investment in specific therapeutic areas and indications, and identify disease areas that might be struggling for early-stage venture equity financing. The study encompasses $38 billion of venture capital into more than 1,200 U.S. drug companies, receiving more than 2,000 rounds of funding over the last 10 years from 2004-2013.

Key findings include:

Seventy-eight percent of U.S. venture investment for therapeutics went toward novel drug R&D, suggesting innovation is a core priority for venture investors

Total venture funding of drug R&D dropped 21 percent, from $21.5 billion to $16.7 billion, comparing five-year periods before and after the recent financial crisis (2004-2008 vs. 2009-2013)

Disease areas affecting large populations – Diabetes, Psychiatry, Gastrointestinal, Respiratory, Cardiovascular – have seen a decline in novel drug R&D venture funding

Rare disease funding has seen a large increase over the past decade in terms of both dollars raised and number of companies funded

There are fewer first-time Series A financings in recent years, down 30 percent from a peak in 2006, but higher dollar amounts for novel drug R&D venture funding

The BIO Industry Analysis team will release study findings at the 2015 BIO CEO & Investor Conference, taking place February 9-10 in New York City. Conference attendees will have early access to the full report, including presentation of the data and a panel discussion with seasoned venture capitalists.

https://www.bio.org/sites/default/files/BIO-Whitepaper-FINAL.PDF

Rising health care costs have long been considered a significant threat to the American economy, but data released by the Congressional Budget Office (CBO) last month fortunately reveals a surprising reality: the projections for the rate of annual health care expenditures have been revised to reflect lower-than-expected long-term health care costs. Simply put, the fastest-growing part of the federal budget spending on health care programs has slowed sharply. The CBO now estimates that Medicare spending will drop by $49 billion (less than 1%) between 2015 and 2024, while Medicaid spending is expected to drop by $40 billion (approximately 1%) over the next decade. Generic medicines remain a key part of this slow-down in health care spending.

As this year’s generic drug savings report shows, increased reliance by American patients and payors on generic pharmaceuticals, which provide the same medicine and same outcomes for patients at a lower price, is well-placed, saving federal and state programs, consumers, taxpayers, businesses and others $239 billion in 2013 alone. This savings represents a 14% increase over cost savings achieved in 2012.

IMS Institute for Healthcare Informatics’ report shows a tremendous impact on health costs in 2013, with the savings number continuing to increase year-over-year, and a growing savings number for the last 10 years, as well.

Key findings from this year’s Sixth Annual Generic Drug Savings in the U.S. report include:

• Cost savings in 2013 alone reached $239 billion, representing a 14% increase over cost savings achieved in 2012.
• Generic products saved the U.S. health system nearly $1.5 trillion over the past 10 years (2004- 2013).
• Nervous system and cardiovascular treatments accounted for more than half (57%) of cost savings, nearly $140 billion, in 2013.

In addition, the report examines the therapeutic areas in which generics delivered the highest level of savings. In 2013, patients across the spectrum benefitted from the availability of safe, affordable generic drugs, but this year'sreport shows that some therapeutic areas deliver a largerproportion of the savings than others. With 57% of savings derived from cardiovascular and nervous system conditions, these areas dominate the proportion of generic savings. But a closer look shows that metabolic drugs (14%) and anti-infectives (7%) also play a significant role.

Finally, the report confirms that the story of generic savings remains one of growth. In addition to the yearly growth of the savings totals, the report reveals that new generic products coming to market in 2013 saved the U.S. health system $140 billion alone. Established generic products continue to providecost savings and, in 2013, they accounted for $98 billion in savings.

This year's cost savings report coincides with the 30th anniversary of the Drug Price Competition and Patent Term Restoration Act of 1984, more commonly known as the Hatch-Waxman Act. Thirty years ago this month, this legislation was signed into law, creating a successful balance between pharmaceutical innovation and competition and paving the way for access to generic choices formillions of Americans.

With more than 1,500 brand and 1,600 generic medicines available today, the Hatch-Waxman legislation can be credited with encouraging the development of new breakthroughs while spurring competition, lowering prices, and generating trillions of dollars of savings to the American health care system. And because 86% of prescriptions dispensed in the United States are now generic, more than ever, safe and effective generic treatment options can be found in every health care setting, including hospitals, pharmacies, and the medicine cabinets of millions of Americans.

The 30th anniversary of the law represents both a milestone and a pivot point for generic medicines. The industry has a remarkable track record. There is no doubt that safe, effective and affordable generic medicines have helped millions of patients and saved trillions of dollars. But what lies ahead is even more exciting. The coming years hold the promise to deliver increased access and more advanced technology to the patients who need it most, as the next wave of lower-cost medicines for Americans, known as biosimilars, emerge. With FDA's first acceptance of the application for a filing of a biosimilar therapy this year, the next decade promise to show major cost savings from biosimilars, with the potential to reach more than $250 billion over 10 years, according to Express Scripts.

http://www.gphaonline.org/media/cms/GPhA_Savings_Report.9.10.14_FINAL.pdf

National Bioscience Report Shows Industry Robust with Strong Prospects for Growth

A study released today analyzing the U.S. bioscience industry growth over the last eleven years, through the recent recession and early economic recovery, reveals positive trends. The industry demonstrated a strong record of growth from 2001-2012, has navigated the deep economic recession better than most industries and is once again growing.

The report, Battelle/BIO State Bioscience Jobs, Investments and Innovation 2014, the sixth in a biennial series from Battelle and BIO tracking the U.S. bioscience industry, reveals a robust bioscience sector that has weathered difficult economic conditions and is on a course for continued growth. The state-by-state industry assessment finds U.S. bioscience firms directly employ 1.62 million people, a figure that includes nearly 111,000 new, high-paying jobs created since 2001.

Within the private sector, the bioscience industry has been a signature performer over this period, contributing an additional 6.24 million jobs through the indirect employment effect, yielding a total employment impact of 7.86 million jobs. Furthermore, the bioscience industry continues to create and sustain high-wage jobs, paying an average 80% more than the overall private sector average salary – and growing at a faster rate.

The U.S. bioscience industry weathered the recession much better than the overall economy and other leading knowledge-based industries. While national private sector employment fell by 3.1% from the outset of the recession in 2007 through 2012, bioscience industry employment fell a mere 0.4%. While employment has almost returned to

its pre-recession level, the economic output of the bioscience industry has expanded significantly with, 17% growth since 2007, almost twice the national private sector nominal output growth.

The study presents the most up-to-date data available on national, state and metropolitan area bioscience industry employment and recent trends from 2001 to 2012, though much of the assessment focuses on the industry's more recent performance from 2007. The report also presents a set of key bioscience performance metrics and profiles recent university and state initiatives designed to accelerate growth of the biosciences and other technology industries.

"This report highlights the long-term expansion of our industry and the significant impact of the high-paying jobs that come with developing the innovative technologies that are helping feed, fuel and heal the world. These biotech jobs are a critical economic component to states and local communities across the nation," said Jim Greenwood, President and CEO of BIO. "While the bioscience industry has continued to grow, our analysis shows it is not immune to market realties. State-level legislative and regulatory policies directly impact the innovation that brings research from the lab to the marketplace, and BIO will continue to advocate for effective public policy at every level of government."

"The fact that the biosciences industry weathered the recession and slow economic recovery better than the overall economy and has been a top-performing industry over the last decade demonstrates its importance as an economic driver for our nation," said Mitch Horowitz, Vice President at Battelle. "While bioscience employment has picked up in 2011 and 2012, its promising growth cannot be taken for granted."

Additional highlights from the industry analysis include:

The research, testing and medical laboratories subsector has been the primary engine of bioscience industry job growth—increasing employment by 28 percent since 2001 and by nearly 10 percent since 2007.

Through strong economic multiplier impacts, each bioscience job generates an additional 3.9 jobs in the U.S. economy (employment multiplier of 4.9).

The industry is well distributed across the United States and plays a major role as an economic driver. Thirty-three states and Puerto Rico have an employment specialization (20 percent or more concentrated than the nation) in at least one of the five bioscience subsectors, which include agricultural feedstock and chemicals; bioscience-related distribution; drugs and pharmaceuticals; medical devices and equipment; and research, testing and medical labs.

In the recent 2007 to 2012 period, which includes the recession and early years of the recovery, 28 states had overall job gains in the biosciences.

Of the nation's 381 metropolitan areas, 216 have a specialized employment concentration in at least one of the bioscience subsectors. Twenty-nine regions have a specialized employment concentration in at least three of the five subsectors.

Highlights from the analysis of innovation performance metrics include:

The number of bioscience-related patents issued in the U.S. has steadily increased every year since 2009, with a growth rate that has exceeded that for all patents.

Academic R&D in the biosciences saw a $4.7 billion increase from 2009 through 2012 of $4.7 billion, or 14% increase.

Battelle/BIO see signs of stress in the bioscience innovation ecosystem that are a concern for the future if not addressed. These include declines in federal funding for biomedical research through the National Institutes of Health (NIH), as well as recent declines in risk capital invested in the biosciences. These trends can create barriers to advancing bioscience innovation in the future.

http://www.battelle.org/media/press-releases/national-bioscience-report

Over the past decade, the bioscience industry in the U.S. grew by about 110,000 jobs, or more than 7.4 percent, in comparison to a 1-percent increase in employment in all private sector industries. Jobs for biochemists and biophysicists are expected to grow by 19 percent through 2022, faster than the U.S. average of 11 percent, while pharmaceutical and medical manufacturing is driving demand for biological technicians. The demand for forensic science technicians will grow by 20 percent through 2018, and there will be greater opportunities for food scientists and technologists owing to efforts to improve the food supply and food safety.

Annual earnings for those employed in the biosciences averaged more than $88,000 in 2012, well above the national average. Those employed working with agricultural feedstock and chemicals or medical devices and equipment made more than $75,000, while earnings were more than $100,000 in the drugs and pharmaceuticals sub-sector.

Most jobs in the biosciences require some postsecondary education. Occupations in this sector require academic, technical and employability skills, not only for research, development and manufacturing but also in bioinformatics; law and regulatory affairs; quality control and sales; marketing and business management; and more. In addition, a number of jobs in other industries make use of bioscience products and processes. Here is just a small sampling of bioscience occupations:

pharmaceutical sales representatives

agricultural and food science technicians

market research analysts

biomedical engineers

forensic science technicians

medical equipment wholesalers

first-line supervisors for medical instrument manufacturing

bioinformatic scientists

https://www.acteonline.org/uploadedFiles/Assets_and_Documents/Global/files/Publications/Sector_Sheet_Biosciences.pdf

Leading Companies

Alexion Pharmaceuticals (NASDAQ: ALXN)

Alexion Reports Second Quarter 2016 Results

- Total Revenues of $753 Million; Increased 18 Percent Year-on-Year; 23 Percent Volume Increase Year-on-Year-

- Soliris® (eculizumab) Revenue Growth Driven by Steady Number of New Patients with PNH and aHUS Treated Globally -

- Strong Strensiq® (asfotase alfa) Launch Continues in Initial Countries -

- Kanuma® (sebelipase alfa) Launch Progresses with Newly Identified Patients Starting on Treatment -

- Eculizumab Phase 3 REGAIN Data in Refractory gMG Presented at the ICNMD Congress -

- ALXN1210 Phase 1/2 Data Showed Rapid and Sustained Reductions in LDH in All Patients with PNH Treated with Once-Monthly Dosing -

- SBC-103 Phase 1/2 Data on MRI and Neurocognitive Assessments Consistent With Potential Dose-Dependent Disease Stabilization at Six Months in Patients with MPS IIIB -

- GAAP EPS of $0.51 Per Share; Non-GAAP EPS of $1.13 Per Share, Which Reflects a Reduction of $0.12 Per Share Attributable to the Modification of Reported Non-GAAP Income Tax Expense –

Thursday, July 28, 2016 6:30 am EDT

NEW HAVEN, Conn. - (BUSINESS WIRE) - Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN) today announced financial results for the second quarter of 2016. Total revenues grew to $753 million, an 18 percent increase, compared to $636 million for the same period in 2015. In the second quarter, the negative impact of currency on total revenue was 3 percent or $18 million, net of hedging activities, compared to the same quarter last year. On a GAAP basis, diluted earnings per share (EPS) for the second quarter of 2016 was $0.51 per share, compared to $0.83 per share in the second quarter of 2015. Non-GAAP diluted EPS for the second quarter 2016 was $1.13 per share, reflecting a reduction of $0.12 per share attributable to the modification of reported non-GAAP income tax expense; prior to this modification non-GAAP diluted EPS would have been reported at $1.25 per share (Table 2). Non-GAAP diluted EPS was $1.30 per share in the second quarter 2015, reflecting a reduction of $0.14 per share attributable to the tax modification.

Alexion has modified the definition of its non-GAAP income tax expense to align with the Compliance & Disclosure Interpretations (C&DIs) issued by the U.S. Securities and Exchange Commission (SEC) on May 17, 2016, and has reflected this modification in 2015 and 2016 non-GAAP interim period results. Alexion's modified definition no longer includes the cash tax benefits the Company realizes during the year from net operating losses and income tax credits, and now includes other deferred taxes. The modification does not change the amount of cash taxes the Company will pay in 2016, or in the future, or have any impact on cash flow. A reconciliation of GAAP to non-GAAP financial results (Table 2) and supplemental effective tax rate information for financial guidance (Table 6) are provided later in the press release.

"In Q2 2016, we delivered strong financial performance as we served an increasing number of patients with PNH, aHUS, HPP and LAL-D. We are pleased with the sustained growth in our core Soliris business, the strong launch of Strensiq, and the continued progress with our Kanuma launch," said David Hallal, Chief Executive Officer of Alexion. "In the second half of 2016, we will continue to leverage our rare disease expertise to reach more patients with Soliris, Strensiq and Kanuma while advancing multiple milestones in our robust pipeline."

Second Quarter 2016 Financial Highlights

- Soliris® (eculizumab) net product sales were $701 million, compared to $636 million in Q2 2015, representing a 10 percent increase. Soliris volume increased 15 percent year-on-year.
- Strensiq® (asfotase alfa) net product sales were $45 million.
- Kanuma® (sebelipase alfa) net product sales were $6 million.
- GAAP R&D expense was $179 million, compared to $132 million in the same quarter last year. Non-GAAP R&D expense was $165 million, compared to $117 million in the same quarter last year.
- GAAP SG&A expense was $232 million, compared to $221 million in the same quarter last year. Non-GAAP SG&A expense was $200 million, compared to $169 million in the same quarter last year.
- GAAP diluted EPS was $0.51 per share, compared to $0.83 per share in the same quarter last year. Non-GAAP diluted EPS was $1.13 per share, reflecting a reduction of $0.12 per share attributable to the modification of reported non-GAAP income tax expense, compared to $1.30 per share, reflecting a reduction of $0.14 per share attributable to the modification of non-GAAP income tax expense in the same quarter last year. GAAP and non-GAAP EPS in the second quarter of 2016 includes the impact of a full quarter of Synageva operations, shares issued for the acquisition and interest expense on related borrowings.

Product and Pipeline Updates

Complement Portfolio

- Eculizumab- Refractory Generalized Myasthenia Gravis (gMG): Data from the REGAIN study, a single, multinational, placebo-controlled Phase 3 trial of eculizumab in patients with refractory gMG, were presented at the International Congress on Neuromuscular Diseases (ICNMD) meeting. Alexion expects to provide an update on discussions with regulators by the end of the year.
- Eculizumab- Relapsing Neuromyelitis Optica Spectrum Disorder (NMOSD): Alexion expects to complete enrollment this year in the PREVENT study, a single, multinational, placebo-controlled Phase 3 trial of eculizumab in patients with relapsing NMOSD.
- Eculizumab- Delayed Graft Function (DGF): Enrollment is complete in the PROTECT study, a single, multinational, placebo-controlled Phase 3 trial of eculizumab in the prevention of DGF, and data are expected in the second half of 2016.
- ALXN1210: New data from the Phase 1/2 study of ALXN1210, a highly innovative longer-acting C5 antibody, in patients with paroxysmal nocturnal hemoglobinuria (PNH) were presented at the European Hematology Association (EHA) Congress. Alexion expects to present additional PNH data later this year. Alexion also expects to initiate a clinical program with ALXN1210 in patients with atypical hemolytic uremic syndrome (aHUS) later this year. The European Commission granted Orphan Drug Designation (ODD) to ALXN1210 for the treatment of patients with PNH.
- ALXN1007: New data from the Phase 2 study of ALXN1007, a complement inhibitor that targets C5a, in patients with graft-versus-host disease involving the lower gastrointestinal tract (GI-GVHD) were presented at EHA and Alexion is now evaluating higher doses of ALXN1007 in patients with GI-GVHD.

Metabolic Portfolio

- SBC-103: New Phase 1/2 data of SBC-103, a recombinant form of the NAGLU enzyme, in patients with mucopolysaccharidosis IIIB, or MPS IIIB, were presented at the International Symposium on MPS and Related Diseases meeting. Alexion has now completed the planned dose escalation, with all patients now randomized to either a 5 mg/kg or 10 mg/kg dose. A natural history study to characterize the course of disease progression in patients with MPS IIIB is ongoing.
- cPMP Replacement Therapy (ALXN1101): Alexion is enrolling patients in a pivotal study to evaluate ALXN1101 in neonates with Molybdenum Cofactor Deficiency (MoCD) Type A. A study to characterize the natural history of MoCD type A was completed in Q2.

Preclinical Portfolio

- Alexion has more than 30 diverse preclinical programs across a range of therapeutic modalities, with four of these programs expected to enter the clinic in 2016.

2016 Financial Guidance

Alexion is reiterating its total revenue and Soliris guidance ranges provided on the first quarter of 2016 earnings call on April 28, 2016, and based on the strength of the Strensiq launch is increasing its Metabolic revenue guidance to $200 to $220 million. Alexion is reiterating its non-GAAP operating expense guidance and is updating its non-GAAP tax rate and non-GAAP EPS guidance. Alexion is also issuing 2016 GAAP financial guidance.

2016 financial guidance is as follows:

	GAAP Guidance	Updated Non-GAAP Guidance	Prior Non-GAAP Guidance
Total revenues	$3,050 to $3,100 million	$3,050 to $3,100 million	Low end of $3,050 to $3,100 million
Soliris revenues	$2,835 to $2,875 million	$2,835 to $2,875 million	$2,835 to $2,875 million
Metabolic revenues	$200 to $220 million	$200 to $220 million	$180 to $200 million
Cost of sales	8% to 9%	8% to 9%	8% to 9%
Research and development expense	$708 to $779 million	High end of $650 to $680 million	High end of $650 to $680 million
Selling, general and administrative expense	$883 to $935 million	High end of $760 to $790 million	High end of $760 to $790 million
Interest expense	$100 million	$100 million	$100 million
Effective tax rate	32% to 34%	15.5% to 16.5% (1)	7% to 8%
Earnings per share	$1.91 to $2.26	$4.50 to $4.65	Low end of $5.00 to $5.20
Diluted shares outstanding	228 million	230 million	230 million

About Alexion

Alexion is a biopharmaceutical company focused on serving patients with severe and rare disorders through the innovation, development and commercialization of life-transforming therapeutic products. Alexion is the global leader in complement inhibition and has developed and markets Soliris® (eculizumab) as a treatment for patients with PNH and aHUS, two debilitating, ultra-rare and life-threatening disorders caused by chronic uncontrolled complement activation. Soliris is currently approved in nearly 50 countries for the treatment of PNH, and in nearly 40 countries for the treatment of aHUS. Alexion is evaluating other potential indications for Soliris in additional severe and ultra-rare disorders beyond PNH and aHUS, and is developing other highly innovative biotechnology product candidates across multiple therapeutic areas.

http://news.alexionpharma.com/press-release/financial-news/alexion-reports-second-quarter-2016-results

Amgen (NASDAQ: AMGN)

Amgen Reports Second Quarter 2016 Financial Results

THOUSAND OAKS, Calif., July 27, 2016 /PRNewswire/ - Amgen (NASDAQ:AMGN) today announced financial results for the second quarter of 2016. Key results include:

- Revenues increased 6 percent versus the second quarter of 2015 to $5.7 billion.

  - Product sales grew 5 percent driven by Enbrel® (etanercept), Prolia® (denosumab), KYPROLIS® (carfilzomib) and XGEVA® (denosumab).
- GAAP earnings per share (EPS) increased 15 percent to $2.47 driven by higher revenues and higher operating margins.
  - GAAP operating income increased 15 percent to $2,380 million and GAAP operating margin improved by 3.8 percentage points to 43.5 percent.
- Non-GAAP EPS increased 11 percent to $2.84 driven by higher revenues and higher operating margins.
  - Non-GAAP operating income increased 10 percent to $2,812 million and non-GAAP operating margin improved by 2.6 percentage points to 51.4 percent.
- 2016 total revenues guidance increased to $22.5-$22.8 billion; EPS guidance increased to $9.55-$9.90 on a GAAP basis and $11.10-$11.40 on a non-GAAP basis.
- The Company generated $2.5 billion of free cash flow.

"We delivered another strong quarter and are on track to meet or exceed our long-term objectives," said Robert A. Bradway, chairman and chief executive officer. "We are in the early stages of a new product launch cycle and have several additional pipeline opportunities rapidly nearing regulatory milestones."

$Millions, except EPS and percentages	Q2'16	Q2'15	YOY Δ
Total Revenues	$ 5,688	$ 5,370	6%
GAAP Operating Income	$ 2,380	$ 2,076	15%
GAAP Net Income	$ 1,870	$ 1,653	13%
GAAP EPS	$ 2.47	$ 2.15	15%
Non-GAAP Operating Income	$ 2,812	$ 2,551	10%
Non-GAAP Net Income	$ 2,146	$ 1,977	9%
Non-GAAP EPS	$ 2.84	$ 2.57	11%

Product Sales Performance

- Total product sales increased 5 percent for the second quarter of 2016 versus the second quarter of 2015. The increase was driven by ENBREL, Prolia, KYPROLIS and XGEVA.
- ENBREL sales increased 10 percent driven by net selling price, offset partially by the impact of competition.
- Neulasta® (pegfilgrastim) sales decreased 1 percent driven by lower unit demand, offset partially by net selling price in the United States (U.S.).
- Aranesp® (darbepoetin alfa) sales increased 5 percent. Unit demand grew due to a shift by some U.S. dialysis customers from EPOGEN® (epoetin alfa) to Aranesp. Unit demand growth was offset partially by unfavorable changes in inventory and net selling price.
- Prolia sales increased 30 percent driven by higher unit demand.
- Sensipar/Mimpara® (cinacalcet) sales increased 13 percent driven by net selling price and higher unit demand.
- XGEVA sales increased 15 percent driven mainly by higher unit demand and, to a lesser extent, net selling price.
- EPOGEN sales decreased 33 percent driven by the impact of competition and, to a lesser extent, a shift by some U.S. dialysis customers to Aranesp.
- NEUPOGEN® (filgrastim) sales decreased 23 percent driven by the impact of competition in the U.S.
- KYPROLIS sales increased 45 percent driven by higher unit demand.
- Vectibix® (panitumumab) sales were flat.
- Nplate® (romiplostim) sales increased 14 percent driven by higher unit demand.
- BLINCYTO® (blinatumomab) sales increased 76 percent driven by higher unit demand.

PRODUCT SALES DETAIL BY PRODUCT AND GEOGRAPHIC REGION

$Millions, except percentages	Q2'16			Q2'15	YOY Δ
	US	ROW	TOTAL	TOTAL	TOTAL
Enbrel®	$1,423	$61	$1,484	$1,348	10%
Neulasta®	962	187	1,149	1,158	(1%)
Aranesp®	260	244	504	479	5%
Prolia®	286	155	441	340	30%
Sensipar® / Mimpara®	303	86	389	344	13%
XGEVA®	275	106	381	331	15%
EPOGEN®	331	0	331	491	(33%)
NEUPOGEN®	141	55	196	256	(23%)
KYPROLIS®	142	30	172	119	45%
Vectibix®	52	108	160	160	0%
Nplate®	84	58	142	125	14%
BLINCYTO®	21	9	30	17	76%
Repatha®	20	7	27	0	*
Other**	17	51	68	57	19%
Total product sales	$4,317	$1,157	$5,474	$5,225	5%

* Not meaningful

** Other includes MN Pharma, Bergamo, IMLYGIC® and Corlanor®

Operating Expense, Operating Margin and Tax Rate Analysis

On a GAAP basis:

- Cost of Sales margin improved by 1.6 percentage points driven primarily by manufacturing efficiencies and higher net selling price. Research & Development (R&D) expenses decreased 7 percent driven primarily by transformation and process improvement efforts and lower spending required to support certain later-stage clinical programs.Selling, General & Administrative (SG&A) expenses increased 11 percent driven primarily by investments in new product launches. Total Operating Expenses were flat year-over-year, with all expense categories reflecting savings from our transformation and process improvement efforts.
- Operating Margin improved by 3.8 percentage points to 43.5 percent.
- Tax Rate decreased by 2.0 percentage points, reflecting discrete benefits associated with tax incentives and the adoption of Accounting Standards Update 2016-09, *Improvements to Employee Share-Based Payment Accounting*(ASU 2016-09), offset partially by unfavorable changes in the geographic mix of earnings.

On a non-GAAP basis:

- Cost of Sales margin improved by 1.6 percentage points driven primarily by manufacturing efficiencies and higher net selling price. R&D expenses decreased 4 percent driven primarily by transformation and process improvement efforts and lower spending required to support certain later-stage clinical programs. SG&A expenses increased 13 percent driven primarily by investments in new product launches. Total Operating Expenses increased 2 percent, with all expense categories reflecting savings from our transformation and process improvement efforts.
- Operating Margin improved by 2.6 percentage points to 51.4 percent.
- Tax Rate decreased by 1.4 percentage points, reflecting discrete benefits associated with tax incentives and the adoption of ASU 2016-09, offset partially by unfavorable changes in the geographic mix of earnings.

$Millions, except percentages	GAAP			Non-GAAP		
	Q2'16	Q2'15	YOY Δ	Q2'16	Q2'15	YOY Δ
Cost of Sales	$1,050	$1,089	(4%)	$738	$789	(6%)
% of product sales	19.2%	20.8%	(1.6) pts	13.5%	15.1%	(1.6) pts

Research & Development	$900	$964	(7%)	$878	$918	(4%)
% of product sales	16.4%	18.4%	(2.0) pts	16.0%	17.6%	(1.6) pts
Selling, General & Administrative	$1,292	$1,160	11%	$1,260	$1,112	13%
% of product sales	23.6%	22.2%	1.4 pts	23.0%	21.3%	1.7 pts
Other	$66	$81	(19%)	$0	$0	0%
TOTAL Operating Expenses	$3,308	$3,294	0%	$2,876	$2,819	2%
Operating Margin						
operating income as a % of product sales	43.5%	39.7%	3.8 pts	51.4%	48.8%	2.6 pts
Tax Rate	15.2%	17.2%	(2.0) pts	18.6%	20.0%	(1.4) pts

pts: percentage points

Cash Flow and Balance Sheet

- The Company generated $2.5 billion of free cash flow in the second quarter of 2016 versus $3.2 billion in the second quarter of 2015. The decrease was driven by the timing of tax payments and the termination of foreign exchange forward contracts in the second quarter of 2015.
- The Company's third quarter 2016 dividend of $1.00 per share declared on July 22, 2016, will be paid on Sept. 8, 2016, to all stockholders of record as of Aug. 17, 2016.
- During the second quarter, the Company repurchased 3.9 million shares of common stock at a total cost of $591 million. At the end of the second quarter, the Company had $3.6 billion remaining under its stock repurchase authorization.

$Billions, except shares	Q2'16	Q2'15	YOY Δ
Operating Cash Flow	$2.7	$3.3	($0.6)
Capital Expenditures	0.2	0.1	0.1
Free Cash Flow	2.5	3.2	(0.7)
Dividends Paid	0.8	0.6	0.2
Share Repurchase	0.6	0.5	0.1
Avg. Diluted Shares (millions)	756	768	(12)
Cash and Investments	35.0	30.0	5.0
Debt Outstanding	33.2	32.0	1.2
Stockholders' Equity	30.1	27.5	2.6

Note: Numbers may not add due to rounding

2016 Guidance

For the full year 2016, the Company now expects:

- Total revenues in the range of $22.5 billion to $22.8 billion.
  - Previously, the Company expected total revenues in the range of $22.2 billion to $22.6 billion.
- On a GAAP basis, EPS in the range of $9.55 to $9.90 and a tax rate in the range of 16.5 percent to 17.5 percent.
  - Previously, the Company expected GAAP EPS in the range of $9.34 to $9.74. Tax rate guidance is unchanged.
- On a non-GAAP basis, EPS in the range of $11.10 to $11.40 and a tax rate in the range of 19.0 percent to 20.0 percent.
  - Previously, the Company expected non-GAAP EPS in the range of $10.85 to $11.20. Tax rate guidance is unchanged.
- Capital expenditures to be approximately $700 million.

SECOND QUARTER PRODUCT AND PIPELINE UPDATE

Key development milestones:

Clinical Program	Indication	Milestone
Repatha® (evolocumab)	Hyperlipidemia	Phase 3 coronary imaging data expected H2 2016 Phase 3 CV outcomes data expected Q1 2017*
KYPROLIS	Newly diagnosed multiple myeloma	Phase 3 data expected H2 2016*
BLINCYTO®	Pediatric Ph- R/R B-cell precursor ALL	FDA priority review
Parsabiv™ (etelcalcetide)†	Secondary hyperparathyroidism	Global regulatory reviews
XGEVA	Prevention of SREs in multiple myeloma	Phase 3 data expected H2 2016*
Romosozumab	Postmenopausal osteoporosis	US regulatory review Global regulatory submissions
Erenumab (AMG 334)	Migraine Prophylaxis	Phase 3 episodic migraine data expected H2 2016
ABP 215 (biosimilar bevacizumab)	Oncology	Global regulatory submissions
ABP 501 (biosimilar adalimumab)	Inflammatory diseases	Global regulatory reviews
ABP 980 (biosimilar trastuzumab)	Breast Cancer	Global regulatory submissions

The Company provided the following updates on selected product and pipeline programs:

Repatha

- In July, the U.S. Food and Drug Administration (FDA) approved the Repatha *Pushtronex*™ system (on-body infusor with prefilled cartridge) for monthly single-dose administration.
- Data from a Phase 3 study evaluating the effects of Repatha on atherosclerotic disease as measured by intravascular ultrasound are expected in H2 2016.
- Data from an event driven Phase 3 study evaluating the effects of Repatha on cardiovascular outcomes are expected in Q1 2017.

KYPROLIS

- In June, the European Commission approved an expanded indication for KYPROLIS, to be used in combination with dexamethasone alone, for adult patients with multiple myeloma who have received at least one prior therapy, based on the ENDEAVOR data.
- Data from the event driven Phase 3 CLARION study of KYPROLIS versus bortezomib in newly diagnosed, transplant ineligible multiple myeloma patients is expected in H2 2016.

BLINCYTO

- In May, FDA accepted for priority review the supplemental Biologics License Application for BLINCYTO to include new data supporting the treatment of pediatric and adolescent patients with Philadelphia chromosome-negative relapsed or refractory B-cell precursor acute lymphoblastic leukemia. The Prescription Drug User Fee Act target action date is Sept. 1, 2016.

Romosozumab

- In July, a Biologics License Application for romosozumab for the treatment of osteoporosis in postmenopausal women at increased risk for fracture was submitted to FDA.

Erenumab

- In June, a global Phase 2 study evaluating the efficacy and safety of erenumab in chronic migraine prevention met its primary endpoint.

ABP 980

- In July, the primary analysis was completed for a Phase 3 study evaluating the efficacy and safety of ABP 980 compared with trastuzumab in patients with human epidermal growth factor receptor 2-positive early breast cancer.

About Amgen

Amgen is committed to unlocking the potential of biology for patients suffering from serious illnesses by discovering, developing, manufacturing and delivering innovative human therapeutics. This approach begins by using tools like advanced human genetics to unravel the complexities of disease and understand the fundamentals of human biology.

Amgen focuses on areas of high unmet medical need and leverages its biologics manufacturing expertise to strive for solutions that improve health outcomes and dramatically improve people's lives. A biotechnology pioneer since 1980, Amgen has grown to be one of the world's leading independent biotechnology companies, has reached millions of patients around the world and is developing a pipeline of medicines with breakaway potential.

http://www.amgen.com/media/news-releases/2016/07/amgen-reports-second-quarter-2016-financial-results/

Biogen Idec (NASDAQ: BIIB)

Biogen Reports Second Quarter 2016 Revenues of $2.9 Billion

Second quarter 2016 GAAP diluted EPS rise 22%; Non-GAAP diluted EPS rise 23%

Company raises financial guidance for the year and authorizes $5 billion share repurchase program

George A. Scangos, Ph.D., to step down as CEO

Thursday, July 21, 2016 7:00 am EDT

CAMBRIDGE, Mass. - (BUSINESS WIRE) - Biogen Inc. (NASDAQ: BIIB) today reported second quarter 2016 financial results, including:

- Total revenues of $2.9 billion, a 12% increase versus the same period in the prior year.
    - Growth was driven by increases in worldwide revenues from the Company's multiple sclerosis (MS) and hemophilia businesses.
    - Foreign exchange negatively impacted total revenues by approximately $44 million compared to the second quarter of 2015, driven by changes in hedge results.
- GAAP net income attributable to Biogen Inc. of $1.0 billion, a 13% increase versus the same quarter in the prior year.
- GAAP diluted earnings per share (EPS) of $4.79, a 22% increase versus the same quarter in the prior year.
- Non-GAAP net income attributable to Biogen Inc. of $1.1 billion, a 15% increase versus the same quarter in the prior year.
- Non-GAAP diluted EPS of $5.21, a 23% increase versus the same quarter in the prior year.

(In millions, except per share amounts)	Q2 '16	Q1 '16	Q2 '15	Q2 '16 v. Q1 '16	Q2 '16 v. Q2 '15
Total revenues	$ 2,894	$ 2,727	$ 2,592	6%	12%
GAAP net income*	$ 1,050	$ 971	$ 927	8%	13%
GAAP diluted EPS	$ 4.79	$ 4.43	$ 3.93	8%	22%

Non-GAAP net income*	$ 1,142	$ 1,049	$ 995	9%	15%
Non-GAAP diluted EPS	$ 5.21	$ 4.79	$ 4.22	9%	23%

*Net income attributable to Biogen Inc.

A reconciliation of GAAP to Non-GAAP quarterly financial results can be found in Table 3 at the end of this release.

“During the second quarter we saw solid performance across our commercial business, as a growing number of patients benefited from our broad MS portfolio, hemophilia therapies, and recently launched biosimilar,” said Chief Executive Officer George A. Scangos, Ph.D. “Revenue strength coupled with thoughtful management of expenses helped drive healthy earnings growth for the quarter. As a result, we have raised our financial guidance for the full year. Our Board has also authorized a $5 billion share repurchase program. We believe this allows us to return capital to shareholders, while leaving ample room for strategic flexibility.”

“We also made important progress for patients with the U.S. and E.U. approvals of ZINBRYTATM and the E.U. approval of FLIXABI®,” Dr. Scangos continued. “And we are excited about our science and research as we shape a robust pipeline of novel candidates we believe could have a significant impact on neurological and related conditions. We continue to enroll two Phase 3 clinical trials for aducanumab in early Alzheimer’s disease; our collaboration partner Ionis Pharmaceuticals has completed enrollment in two Phase 3 studies of nusinersen in infants and children with spinal muscular atrophy; and we have announced an innovative gene therapy collaboration with the University of Pennsylvania focused on potential treatments targeting the central nervous system.”

Expense Highlights

- GAAP cost of sales was $370 million compared to $313 million in the first quarter of 2016 and $286 million in the second quarter of 2015.
- Non-GAAP cost of sales was $354 million compared to $313 million in the first quarter of 2016 and $286 million in the second quarter of 2015.
- GAAP and Non-GAAP R&D expense was $473 million compared to $437 million in the first quarter of 2016 and $491 million in the second quarter of 2015.
- GAAP SG&A expense was $492 million compared to $497 million in the first quarter of 2016 and $492 million in the second quarter of 2015.
- Non-GAAP SG&A expense was $489 million compared to $497 million in the first quarter of 2016 and $492 million in the second quarter of 2015.

Other Financial Highlights

- For the second quarter of 2016, the Company’s weighted average diluted shares were 219 million.
- As of June 30, 2016, Biogen had cash, cash equivalents and marketable securities totaling approximately $7.3 billion, and $6.5 billion in notes payable and other financing arrangements.

Share Repurchase Update

Biogen announced that its Board of Directors authorized a program to repurchase up to $5 billion of the Company’s common stock. Biogen currently expects that purchases will be executed over the next three years. This share repurchase program is in addition to the approximately 1.3 million shares remaining under Biogen's February 2011 share repurchase program, which has been used principally to offset common stock issuances under the Company's share-based compensation plans.

2016 Financial Guidance

Biogen updated its full year 2016 financial guidance. This guidance consists of the following components:

- Revenue is expected to be approximately $11.2 to $11.4 billion.

- GAAP and non-GAAP R&D expense is expected to be approximately 17% to 18% of total revenue.
- GAAP and non-GAAP SG&A expense is expected to be approximately 16% to 17% of total revenue.
- GAAP diluted EPS is expected to be between $18.10 and $18.40.
- Non-GAAP diluted EPS is expected to be between $19.70 and $20.00.

This guidance includes contribution from our hemophilia business through the end of the year, as we now anticipate the spin-off to complete in early 2017. This guidance does not include any impact from potential acquisitions or late-stage business development transactions.

Biogen may incur charges, realize gains or experience other events in 2016 that could cause actual results to vary from this guidance.

CEO Transition

Biogen today announced that George Scangos, its Chief Executive Officer, will be leaving the Company in the coming months after a successor has been identified. The Company will begin a search for his successor immediately. Dr. Scangos has been at Biogen for six years and has led the Company through a remarkable transformation. Under his leadership, Biogen's revenues, earnings and stock price all have increased meaningfully and the Company has been transformed into a world-class biopharmaceutical company.

Stelios Papadopoulos, Chairman of the Biogen Board of Directors, remarked "George joined Biogen at a very challenging time. He re-organized operations and he oversaw the enrichment of our product pipeline and the launch of several products. In short, George did an outstanding job and I believe he is leaving the Company well positioned for success."

"The past six years have been quite successful," said Dr. Scangos. "We have introduced six new products onto the market, increased our earnings and revenues several fold, and transformed our R&D and commercial organizations to world-class levels, joining our already industry leading biologics manufacturing capabilities. We have brought several potentially transformative compounds into later stage clinical development and are in the process of adding to that pipeline even further."

"The Company has an exciting future and I am proud to have had a role in helping Biogen improve the lives of so many patients today and so many more in the future," added Dr. Scangos. "This is the right time for a new leader to take the reins and lead Biogen through its next stage of development, and I look forward to returning to the West coast to take on one more set of activities and spend more time with my family."

The Board will immediately begin a search for a replacement, and will consider both internal and external candidates. The Company expects the transition to occur over a period of a few months, and in the interim, Dr. Scangos will continue to serve as CEO.

Other Recent Events

- In July 2016, the Marketing Authorization Application (MAA) for SB5, an adalimumab biosimilar candidate referencing Humira®, was accepted for review by the European Medicines Agency (EMA). The MAA for SB5 is the third anti-TNF biosimilar candidate to be submitted to the EMA by Samsung Bioepis, the joint venture between Samsung BioLogics and Biogen. The approval of SB5 could make Biogen the first company to commercialize three anti-TNF biosimilar therapies in Europe.
- In July 2016, the Roche Group announced that the Phase 3 GOYA study evaluating GAZYVA® plus CHOP chemotherapy in people with previously untreated diffuse large B-cell lymphoma did not meet its primary endpoint of significantly reducing the risk of disease worsening or death (progression-free survival) compared to RITUXAN® plus CHOP chemotherapy. In the U.S., Biogen shares operating profits and losses relating to GAZYVA with Genentech, a Roche Group company.

- In July 2016, Biogen and AbbVie announced that the European Commission (EC) granted marketing authorization for ZINBRYTA for the treatment of adult patients with relapsing forms of MS (RMS). ZINBRYTA is a once-monthly, self-administered, subcutaneous treatment for RMS which has demonstrated superior efficacy to AVONEX (interferon beta-1a).
- In June 2016, the EC approved a variation to the marketing authorization of TYSABRI, which extended its indication to include relapsing-remitting multiple sclerosis patients with highly active disease despite a full and adequate course of treatment with at least one disease modifying therapy. TYSABRI was previously only indicated for patients who had failed to respond to beta-interferon or glatiramer acetate in the European Union (EU). This follows recent EC approval for a new patient management plan including an updated risk algorithm based on JC virus antibody index values.
- In June 2016, the Roche Group announced that the EMA has validated the company's MAA of OCREVUS™ (ocrelizumab) for the treatment of RMS and primary progressive multiple sclerosis (PPMS) in the EU. The U.S. Food and Drug Administration (FDA) has also accepted for review Genentech's Biologics License Application for OCREVUS for the treatment of RMS and PPMS, and has granted the application Priority Review Designation with a targeted action date of 28 December 2016. If approved for commercial sale, Biogen will receive tiered royalties on sales of OCREVUS.
- In June 2016, Biogen announced the appointment of Paul McKenzie, Ph.D., as Executive Vice President, Pharmaceutical Operations & Technology. Dr. McKenzie was previously Senior Vice President of Global Biologics Manufacturing and Technical Operations. He replaces John Cox, who was named Chief Executive Officer of the new Biogen spin-off company.
- In June 2016, Biogen reported top-line results from the Phase 2 SYNERGY study evaluating opicinumab (anti-LINGO-1), an investigational, fully human monoclonal antibody being developed as a potential neuroreparative therapy in people with RMS. In the study, opicinumab missed the primary and secondary endpoints. However, evidence of a clinical effect with a complex, unexpected dose-response was observed. The Company continues to analyze results to determine the appropriate next steps. The Company plans to present results from the SYNERGY study at the 32nd Congress of the European Committee for Treatment and Research in Multiple Sclerosis (ECTRIMS) in September 2016.
- In June 2016, Biogen announced that aducanumab, its investigational treatment for early Alzheimer's disease, was accepted into the PRIority MEdicines (PRIME) program of the EMA. PRIME aims to bring treatments to patients faster by enhancing the EMA's support for the development of investigational medicines for diseases without available treatment or in need of better treatment options.
- In May 2016, Samsung Bioepis, the joint venture between Biogen and Samsung BioLogics, received marketing authorization in the EU for FLIXABI, an infliximab biosimilar referencing Remicade®. FLIXABI is the second anti-TNF biosimilar to be manufactured and commercialized by Biogen in the EU.
- In May 2016, Biogen and AbbVie announced that the FDA approved ZINBRYTA, a new once-monthly, self-administered, subcutaneous treatment for RMS. According to the U.S. prescribing information, because of its safety profile, the use of ZINBRYTA should generally be reserved for patients who have had an inadequate response to two or more therapies indicated for the treatment of MS.
- In May 2016, the Roche Group announced that the Phase 3 GALLIUM study met its primary endpoint early, demonstrating superior progression-free survival for GAZYVA compared to RITUXAN in people with previously untreated follicular lymphoma. Follicular lymphoma is the most common type of indolent (slow-growing) non-Hodgkin lymphoma (NHL) and accounts for approximately one in five cases of NHL. In the U.S., Biogen shares operating profits and losses relating to GAZYVA with Genentech, a Roche Group company.
- In May 2016, Biogen announced a broad collaboration and alliance with the University of Pennsylvania to advance gene therapy and gene editing technologies, with a primary focus on the development of therapeutic approaches that target the eye, skeletal muscle and the central nervous system. Biogen will work with renowned gene therapy experts, Dr. James Wilson and Dr. Jean Bennett.
- In May 2016, Swedish Orphan Biovitrum AB (publ) (Sobi) and Biogen announced that the EC approved ALPROLIX, an extended half-life recombinant factor IX Fc fusion protein therapy for the treatment of hemophilia B, in the EU.

- In May 2016, Biogen announced its intent to spin off its hemophilia business as an independent, publicly traded company. The new company is expected to continue to commercialize ELOCTATE and ALPROLIX under Biogen's existing collaboration agreement with Sobi, while continuing to engage in ongoing research and development activities to develop longer acting therapies utilizing XTEN® technology, bispecific antibodies, and hemophilia-related gene therapy programs.
- In April 2016, Biogen announced the appointment of Michael Ehlers, M.D., Ph.D. as Executive Vice President, Research and Development. Dr. Ehlers joins Biogen from Pfizer, where he served as Group Senior Vice President for BioTherapeutics R&D and Chief Scientific Officer for the company's Neuroscience and Pain Research Unit.

About Biogen

Through cutting-edge science and medicine, Biogen discovers, develops and delivers to patients worldwide innovative therapies for the treatment of neurodegenerative diseases, hematologic conditions and autoimmune disorders. Founded in 1978, Biogen is the world's oldest independent biotechnology company and patients worldwide benefit from its leading multiple sclerosis and innovative hemophilia therapies

http://media.biogen.com/press-release/investor-relations/biogen-reports-second-quarter-2016-revenues-29-billion

BioMarin Pharmaceutical Inc. (NASDAQ: BMRN)

BioMarin Announces First Quarter 2016 Financial Results

-First Quarter 2016 Total BioMarin Revenue Increases 16.7% Y/Y to $236.7 million

-Vimizim Net Product Revenue Increases 43.5% Y/Y and Contributes $72.6 million in the First Quarter 2016; Vimizim Full-year Revenue Guidance Increased to $315 to $340 million

- Kuvan Net Product Revenue Contributes $76.9 million in the First Quarter 2016; $60.0 million from North America and $16.9 million from Newly Acquired ex-North American Territories

Financial Highlights (in millions of U.S. dollars, except per share data, unaudited)

	Three Months Ended March 31,		
	2016	2015	% Change
Total BioMarin Revenue	$ 236.7	$ 202.9	16.7 %
Vimizim Net Product Revenue	72.6	50.6	43.5 %
Naglazyme Net Product Revenue	65.4	78.2	(16.4)%
Kuvan Net Product Revenue	76.9	50.2	53.2 %
Aldurazyme Net Product Revenue	16.4	18.2	(9.9)%
Non-GAAP Net Loss	$ (27.2)	$ (25.4)	
GAAP Net Loss	$ (85.1)	$ (67.5)	
GAAP Net Loss per Share - Basic and Diluted	$ (0.53)	$ (0.43)	
Cash, cash equivalents and investments	$ 771.3	$ 1,018.3	

SAN RAFAEL, Calif., April 28, 2016 (GLOBE NEWSWIRE) - BioMarin Pharmaceutical Inc. (NASDAQ:BMRN) today announced financial results for the first quarter ended March 31, 2016. Non-GAAP net loss was $27.2 million for the quarter ended March 31, 2016, compared to non-GAAP net loss of $25.4 million for the first quarter of 2015. GAAP net loss was $85.1 million, or $0.53 per basic and diluted share for the first quarter of 2016, compared to GAAP net loss of $67.5 million, or $0.43 per basic and diluted share, for the first quarter of 2015.

Total BioMarin Revenue was $236.7 million for the first quarter of 2016, an increase of 16.7% compared to the same period in 2015. This strong result was driven by year over year growth of 43.5% and 53.2% of Vimizim and Kuvan, respectively. Kuvan revenue from ex-North America territories since BioMarin acquired worldwide rights in January 2016 contributed $16.9 million and revenues in North America contributed $60.0 million in the quarter. Naglazyme patient growth was 8.5% compared to a year ago, the 40th straight quarter since the product was launched in 2005. Naglazyme revenue in the first quarter 2016 was lower than revenue in the first quarter 2015 primarily due to the timing of central government orders from Latin America.

As of March 31, 2016, BioMarin had cash, cash equivalents and investments totaling $771.3 million, as compared to $1,018.3 million on December 31, 2015.

Commenting on the quarter, Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin said, "Our commercial base business is robust and is expected to generate over one billion dollars in revenues this year. Prospects for new product launches in 2017 increased during the quarter due to positive data readouts for cerliponase alfa and pegvaliase that we expect will lead to two new product filings later this year. In addition, at our recent Research and Development Day for analysts and investors, we highlighted very encouraging preliminary data from our gene therapy product BMN 270 for hemophilia A and robust 12 month data with vosoritide for achondroplasia. If the data from these programs continue to mature as we hope, we believe that these products could each ultimately drive a billion dollars in revenue when commercialized. Finally, we continue to expect to manage this growing business with the goal of achieving non-GAAP break-even or better in 2017 regardless of the regulatory outcome of Kyndrisa in Europe."

Net Product Revenue (in millions of U.S. dollars, unaudited)

Total Revenue

	Three Months Ended March 31,			
	2016	2015	$ Change	% Change
Vimizim [1]	$ 72.6	$ 50.6	$ 22.0	43.5 %
Naglazyme [1]	65.4	78.2	(12.8)	(16.4)%
Kuvan [2]	76.9	50.2	26.7	53.2 %
Aldurazyme	16.4	18.2	(1.8)	(9.9)%
Firdapse	4.1	4.1	-	0.0 %
Net product revenues	235.4	201.3	34.1	16.9 %
Collaborative agreement revenues	0.2	0.4	(0.2)	
Royalty, license and other revenues	1.1	1.2	(0.1)	
Total BioMarin revenues	$ 236.7	$ 202.9	$ 33.8	16.7 %

Reconciliation of Aldurazyme Revenues

	Three Months Ended March 31,			
	2016	2015	$ Change	% Change
Aldurazyme revenue reported by Genzyme	$ 52.8	$ 53.4	$ (0.6)	(1.1)%

	Three Months Ended March 31,		
	2016	2015	$ Change
Royalties earned from Genzyme	$ 21.5	$ 22.3	$ (0.8)
Net product transfer revenues [3]	(5.1)	(4.1)	(1.0)
Total Aldurazyme net product revenues	$ 16.4	$ 18.2	$ (1.8)

2016 Financial Guidance

Revenue Guidance ($ in millions)

Item	Provided February 25, 2016	Updated April 28, 2016
Total BioMarin Revenues	$1,050 to $1,100	Unchanged
Vimizim Net Product Revenue	$300 to $330	$315 to $340
Naglazyme Net Product Revenue	$290 to $320	Unchanged
Kuvan Net Product Revenue	$320 to $350	Unchanged

Select Income Statement Guidance ($ in millions, except percentages)

Item	Provided February 25, 2016	Updated April 28, 2016
Cost of Sales (% of Total Revenue)	18.0% to 19.0%	Unchanged
Selling, General and Admin. Expense	$470 to $490	Unchanged
Research and Development Expense	$680 to $720	Unchanged
Non - GAAP Net Loss	$(75) to $(100)	Unchanged
GAAP Net Loss	$(400) to $(430)	$(355) to $(385)*

Key Program Updates at R&D Day April 20, 2016

- BMN 270 gene therapy product for hemophilia A: The Company provided encouraging preliminary data from an ongoing Phase 1/2 clinical trial with BMN 270, an investigational gene therapy treatment for hemophilia A. A total of eight patients with severe hemophilia A received a single dose of BMN 270, six of whom have been treated at the highest dose of 6 x 10^{13} vector genomes (VG)/kilogram (kg), and to date, post-treatment follow-up ranges from five to 16 weeks. As stated at R&D Day, patients at the highest dose experienced increasing Factor VIII activity levels ranging between 4% and 60% (as a percentage of normal

calculated based on the numbers of International Units (IU) per milliliter of whole blood), with five of six patients treated at the high dose now over 5% and two of six at over 50%. All high dose patients improved from severe to either moderate, mild or normal range in terms of factor levels based on World Federation of Hemophilia criteria. (See BioMarin press release from April 20, 2016 for further details.)

- Vosoritide for achondroplasia: The Company provided an update on its Phase 2 study of vosoritide, an analog of C-type Natriuretic Peptide (CNP), in children with achondroplasia, the most common form of dwarfism. After 12 months of daily dosing at 15 µg/kg/day, the cohort 3 patients (n=10) experienced a 46% or 1.9 cm/year increase in mean annualized growth velocity from baseline (p-value = 0.02). These findings provide evidence of durability of effect consistent with previously presented 6-month data for these patients, which demonstrated an annualized increase of 50% or 2.0 cm/year in mean annualized growth velocity. In addition, 6-month data for 12 patients who were initiated on a lower dose and switched to 15 µg/kg/day showed an increase of 65% or 2.3 cm/year in mean annualized growth velocity from baseline (p-value = 0.002). (See BioMarin press release from April 20, 2016 for further details.)

- Cerliponase alfa for CLN2, late-infantile form of Batten disease: Complete results from the Phase 1/2 study of cerliponase alfa, a recombinant human tripeptidyl peptidase 1 (rhTPP1), for the treatment of patients with late-infantile neuronal ceroid lipofuscinosis type 2 (NCL-2), a form of Batten disease were announced at the WORLD LSD Symposium on March 2, 2016. Based on the robust data results announced at that meeting, the Company shared plans to submit in the U.S. and E.U. for regulatory approval mid-year 2016. (See BioMarin press release from March 2, 2016 for further details.)

- Pegvaliase for phenylketonuria (PKU): Pivotal results for the Phase 3 PRISM-2 study (formerly referred to as 165-302) that pegvaliase met the primary endpoint of change in blood Phe compared with placebo ($p<0.0001$) were announced March 21, 2016. The pegvaliase treated group maintained mean blood Phe levels at 527.2 umol/L compared to their RDT baseline of 503.9 umol/L, whereas the placebo treated group mean blood Phe levels increased to 1385.7 umol/L compared to their RDT baseline of 536.0 umol/L. The treatment effect demonstrated in this study represents an approximately 62% improvement in blood Phe compared to placebo. Based on the supportive data results, the Company plans to submit a Biologics License Application (BLA) to U.S. FDA in the second half of 2016. (See BioMarin press release from March 21, 2016 for further details.)

About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises five approved products and multiple clinical and pre-clinical product candidates. Approved products include: Naglazyme® (galsulfase) for mucopolysaccharidosis VI (MPS VI), a product wholly developed and commercialized by BioMarin; Aldurazyme® (laronidase) for mucopolysaccharidosis I (MPS I), a product which BioMarin developed through a 50/50 joint venture with Genzyme Corporation; KUVAN® (sapropterin dihydrochloride) Powder for Oral Solution and Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany; Firdapse® (amifampridine), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS); and VIMIZIM® (elosulfase alfa) for the treatment of Morquio A (MPS IVA). Product candidates include: BMN 165 (PEGylated recombinant phenylalanine ammonia lyase), also referred to as PEG PAL, which is currently in Phase 3 clinical development for the treatment of PKU; talazoparib (BMN 673), a poly ADP-ribose polymerase (PARP) inhibitor, which is currently in Phase 3 clinical development for the treatment of germline BRCA breast cancer; BMN 701, a novel fusion of acid alpha glucosidase (GAA) with a peptide derived from insulin like growth factor 2, which is currently in Phase 3 clinical development for the treatment of Pompe disease; BMN 111, a modified C-natriuretic peptide, which is currently in Phase 2 clinical development for the treatment of achondroplasia; and BMN 190, a recombinant human tripeptidyl peptidase-1 (rhTPP1), which is currently in Phase 1 for the treatment of late-infantile neuronal ceroid lipofuscinosis (CLN2), a form of Batten Disease. For additional information, please visit www.BMRN.com. Information on BioMarin's website is not incorporated by reference into this press release.

http://investors.bmrn.com/releasedetail.cfm?ReleaseID=968019

Celgene Corporation (NASDAQ: CELG)

Celgene Reports Second Quarter 2016 Operating and Financial Results

- Net Product Sales $2.74B in Q2:16: Increased 22% Y/Y
- REVLIMID® Net Product Sales $1.7B in Q2:16; Increased 18% Y/Y
- 2016 Guidance Updated: REVLIMID® and Total Net Product Sales; EPS

Jul 28, 2016

SUMMIT, N.J. - (BUSINESS WIRE) - Celgene Corporation (NASDAQ:CELG) reported net product sales of $2,745 million for the second quarter of 2016, a 22 percent increase from the same period in 2015. Net product sales growth includes a 1 percent negative impact from currency exchange effects. Second quarter total revenue increased 21 percent to $2,754 million compared to $2,278 million in the second quarter of 2015.

Net income for the second quarter of 2016 based on U.S. GAAP (Generally Accepted Accounting Principles), was $598 million or $0.75 per diluted share compared to $356 million or $0.43 per diluted share in the second quarter of 2015. Adjusted net income for the second quarter of 2016 was$1,152 million or $1.44 per diluted share compared to $1,019 million or $1.23 per diluted share for the second quarter of 2015.

"Our first-half 2016 operating results were outstanding and we are pleased with the progress made advancing many key corporate objectives," said Mark J. Alles, Chief Executive Officer of Celgene Corporation. "This strong momentum increases our confidence in our near- and longer-term outlook as we continue to invest in innovative research and the development of transformational therapies for patients worldwide."

Second Quarter 2016 Financial Highlights

Unless otherwise stated, all comparisons are for the second quarter of 2016 compared to the second quarter of 2015. The adjusted operating expense categories presented below exclude share-based employee compensation expense, upfront collaboration expense and a litigation-related loss contingency accrual expense. Please see the attached Reconciliation of GAAP to Adjusted Net Income for further information.

Net Product Sales Performance

- REVLIMID® sales for the second quarter increased 18 percent year-over-year to $1,701 million and were driven by new patient market share gains and increased duration. U.S. sales of $1,080 million and international sales of $621 million increased 24 percent and 9 percent year-over-year, respectively.
- POMALYST®/IMNOVID® sales for the second quarter were $318 million, an increase of 35 percent year-over-year. U.S. sales were $185 million and international sales were $133 million, an increase of 29 percent and 46 percent year-over-year, respectively. POMALYST®/IMNOVID® sales grew due to increased volume from duration gains.
- ABRAXANE® sales for the second quarter were $249 million, a 2 percent increase year-over-year. U.S. sales of $175 million increased 3 percent year-over-year. International sales were $74 million.
- OTEZLA® sales for the second quarter were $242 million, a 170 percent increase year-over-year. U.S. sales were $217 million and international sales were $25 million. Sales were driven by market share gains and increased prescriber adoption.
- In the second quarter, all other product sales, which include THALOMID®, ISTODAX®, VIDAZA® and an authorized generic version of VIDAZA® drug product in the U.S., were $235 million compared to $242 million in the second quarter of 2015.

Research and Development (R&D)

On a GAAP basis, R&D expenses were $949 million for the second quarter of 2016 compared to $1,110 million for the same period in 2015. The change was primarily driven by a decrease in upfront collaboration expenses compared to the previous year, partially offset by early research and clinical trial activity related to the acquisitions of Receptos, Inc. and Quanticel Pharmaceuticals, Inc. that closed in the second half of 2015. Adjusted R&D expenses were $601

million for the second quarter of 2016 compared to $477 million for the second quarter of 2015. Adjusted R&D does not include upfront collaboration expenses but does reflect the increase in early research and clinical trial activity.

Selling, General, and Administrative (SG&A)

On a GAAP basis, SG&A expenses were $732 million for the second quarter of 2016 compared to $617 million for the same period in 2015. The increase was primarily due to a loss contingency accrual expense of $100 million related to a contractual dispute. Adjusted SG&A expenses were$547 million for the second quarter of 2016 compared to $541 million for the second quarter of 2015.

Cash, Cash Equivalents, and Marketable Securities

Operating cash flow was $936 million in the second quarter of 2016. Celgene ended the quarter with approximately $6.4 billion in cash, cash equivalents and marketable securities.

In the second quarter of 2016, Celgene purchased approximately 3.4 million of its shares at a total cost of approximately $343 million. In June 2016, the share repurchase authorization was increased by an additional authorization of $3 billion. As of June 30, 2016, the Company had approximately $5.1 billion remaining under the stock repurchase program.

2016 Guidance Updated

	Previous 2016 Guidance	Updated 2016 Guidance
Net Product Sales		
Total	$10.75B-$11.0B	Approximately $11.0B
REVLIMID®	Approximately $6.7B	Approximately $6.8B
GAAP diluted EPS	$4.26 to $4.56	$3.82 to $4.05
Adjusted diluted EPS	$5.60 to $5.70	$5.70 to $5.75
GAAP operating margin	Approximately 42%	Approximately 37%
Adjusted operating margin	Approximately 53.5%	Approximately 54.0%
Weighted average diluted shares	811M	806M

Net product sales guidance for POMALYST®/IMNOVID®, ABRAXANE® and OTEZLA® remain unchanged.

Product and Pipeline Updates

Hematology/Oncology

- At the American Society of Clinical Oncology (ASCO) meeting in June, pooled data from a meta-analysis of overall survival (OS) in multiple myeloma patients receiving REVLIMID® as maintenance treatment following autologous stem-cell transplant were presented. An application was submitted to the European Medicines Agency (EMA) in early June for the review of REVLIMID® as maintenance treatment in newly diagnosed multiple myeloma (NDMM) patients after receiving an autologous stem-cell transplant. A decision on the application is expected in 2017. A submission in the U.S. is expected in the second half of 2016.
- In July, the European Commission (EC) approved REVLIMID® for the treatment of adult patients with relapsed or refractory mantle cell lymphoma. REVLIMID® is approved in the U.S. for the treatment of mantle cell lymphoma after relapse or progression on two prior therapies.
- In June, the U.S. product insert for POMALYST® was updated to include data from a pooled pharmacokinetics analysis of patients with relapsed and/or refractory multiple myeloma (RRMM) and impaired renal function. In Europe, the Committee for Medicinal Products for Human Use (CHMP) granted a positive opinion for IMNOVID® based on the same data. The EC decision is expected in the third quarter.

- In July, Celgene disclosed the top-line results of the phase III REMARC trial evaluating REVLIMID® as maintenance therapy compared with placebo in patients with diffuse large B-cell lymphoma responding to treatment with rituximab in combination with standard chemotherapy. The full data set will be presented at a future medical congress.
- In July, Celgene's partner Juno Therapeutics provided preliminary data from the ongoing phase I trial with JCAR017 in patients with adult non-Hodgkin lymphoma (NHL). In ten patients evaluable for efficacy, an overall response rate of 80 percent and a complete response rate of 70 percent were seen. In thirteen patients evaluable for safety, the rate of severe neurotoxicity was 15 percent and the rate of cytokine release syndrome was zero percent. An update of the trial data is expected later in the year.
- The FUSION™ program evaluating durvalumab in hematological malignancies continues to advance with six early-stage trials enrolling. The trials are evaluating durvalumab as a single agent or in combination with novel agents in NDMM, RRMM, myelodysplastic syndromes, acute myeloid leukemia, NHL and chronic lymphocytic leukemia.
- A phase II trial with CC-486 in combination with pembrolizumab in previously treated locally advanced or metastatic non-small cell lung cancer completed enrollment in the second quarter.

Inflammation & Immunology

- Long-term data from the PALACE program evaluating OTEZLA® in moderate-to-severe psoriatic arthritis were presented at the EuropeanLeague Against Rheumatism (EULAR) meeting in June. Included was three-year pooled efficacy and safety data from the phase III PALACE program, as well as pooled data on fatigue, HAQ-DI and BASDAI from PALACE 1-3.
- The phase II proof-of-concept trial evaluating OTEZLA® in atopic dermatitis has completed. Celgene is evaluating the data to determine next steps. The data will be published at a later date.
- In May, the phase II TOUCHSTONE trial evaluating ozanimod induction and maintenance in patients with moderate-to-severe ulcerative colitis was published in *The New England Journal of Medicine*. Histologic data from the phase II TOUCHSTONE trial were presented at the Digestive Disease Week meeting in May. The phase III TRUE NORTH trial evaluating ozanimod in patients with moderate-to-severe ulcerative colitis continues to enroll with data expected in 2018.
- The registration-enabling endoscopy trial (CD-001) with GED-0301 in patients with active Crohn's disease completed enrollment. Top-line data from the 12-week portion of the trial is expected in the second half of 2016.

Business Update

- In July, Celgene announced a strategic collaboration with Jounce Therapeutics, Inc. The collaboration includes options on Jounce's lead product candidate, JTX-2011, targeting ICOS (the Inducible T cell CO-Stimulator), and up to four early-stage programs to be selected from a defined pool of B cell, T regulatory cell and tumor-associated macrophage targets emerging from Jounce's research platform, and an additional option on a Jounce checkpoint immuno-oncology program.
- In May, Celgene and Agios Pharmaceuticals, Inc. entered into a new global strategic collaboration for the discovery, development and commercialization of novel metabolic immuno-oncology therapies based on Agios' innovative cellular metabolism research platform. In addition, Celgene transferred global development and commercialization rights to the AG-120 program to Agios.

About Celgene

Celgene Corporation, headquartered in Summit, New Jersey, is an integrated global biopharmaceutical company engaged primarily in the discovery, development and commercialization of innovative therapies for the treatment of cancer and inflammatory diseases through next-generation solutions in protein homeostasis, immuno-oncology, epigenetics, immunology and neuro-inflammation. For more information, please visit www.celgene.com. Follow Celgene on Social Media: @Celgene, Pinterest, LinkedIn, FaceBook and YouTube.

http://ir.celgene.com/releasedetail.cfm?ReleaseID=981642

Gilead Sciences (NASDAQ: GILD)

Gilead Sciences Announces First Quarter 2016 Financial Results

- Product Sales of $7.7 billion -

- GAAP Diluted EPS of $2.53 per share -

- Non-GAAP Diluted EPS of $3.03 per share -

- Reiterates Full Year 2016 Guidance -

OSTER CITY, Calif. - (BUSINESS WIRE) - Apr. 28, 2016 - Gilead Sciences, Inc. (Nasdaq: GILD) announced today its results of operations for the first quarter ended March 31, 2016. The financial results that follow represent a year-over-year comparison of first quarter 2016 to the first quarter 2015. Total revenues were $7.8 billion in 2016 compared to $7.6 billion in 2015. Net income was $3.6 billion or $2.53 per diluted share in 2016 compared to $4.3 billion or $2.76 per diluted share in 2015. Non-GAAP net income, which excludes acquisition-related, up-front collaboration, stock-based compensation and other expenses, was $4.3 billion or $3.03 per diluted share in 2016 compared to $4.6 billion or $2.94 per diluted share in 2015.

	Three Months Ended March 31,	
(In millions, except per share amounts)	2016	2015
Product sales	$ 7,681	$ 7,405
Royalty, contract and other revenues	113	189
Total revenues	$ 7,794	$ 7,594
Net income attributable to Gilead	$ 3,566	$ 4,333
Non-GAAP net income attributable to Gilead	$ 4,274	$ 4,604
Diluted EPS	$ 2.53	$ 2.76
Non-GAAP diluted EPS	$ 3.03	$ 2.94

Product Sales

Total product sales for the first quarter of 2016 were $7.7 billion compared to $7.4 billion for the same period in 2015. Product sales for the first quarter of 2016 were $4.4 billion in the U.S., $1.6 billion in Europe, $1.1 billion in Japan and $571 million in other international locations. Product sales for the first quarter of 2015 were $5.2 billion in the U.S., $1.8 billion in Europe and $364 million in other international locations.

Antiviral Product Sales

Antiviral product sales, which include products in our HIV and liver disease areas, were $7.2 billion for the first quarter of 2016 compared to $7.0 billion for the same period in 2015.

In the U.S., antiviral product sales were $4.0 billion for the first quarter of 2016 compared to $4.9 billion in 2015, primarily due to a decline in sales of Harvoni® (ledipasvir 90 mg/sofosbuvir 400 mg), partially offset by increases in sales of Sovaldi® (sofosbuvir 400 mg), Truvada® (emtricitabine and tenofovir disoproxil fumarate) and Genvoya® (elvitegravir 150 mg/cobicistat 150 mg/emtricitabine 200 mg/tenofovir alafenamide 10 mg). Genvoya was launched in the U.S. in November 2015.

In Europe, antiviral product sales were $1.6 billion for the first quarter of 2016 compared to $1.7 billion in 2015, primarily due to a decline in sales of Sovaldi.

In Japan, antiviral product sales were $1.1 billion. Sovaldi and Harvoni were launched in Japan in May and September 2015, respectively.

Other Product Sales

Other product sales, which include Letairis® (ambrisentan), Ranexa® (ranolazine) and AmBisome® (amphotericin B liposome for injection), were $498 million for the first quarter of 2016 compared to $417 million for the same period in 2015.

Cost of Goods Sold

Non-GAAP* cost of goods sold increased to $983 million for the first quarter of 2016 compared to $674 million for the same period in 2015, primarily due to a $200 million litigation charge related to our sofosbuvir based product sales.

Operating Expenses

Three Months Ended

	March 31,	
(In millions)	2016	2015
Non-GAAP* research and development expenses (R&D)	$ 769	$ 651
Non-GAAP* selling, general and administrative expenses (SG&A)	$ 638	$ 600

During the first quarter of 2016, compared to the same period in 2015:

Non-GAAP research and development expenses increased primarily due to the progression of Gilead's clinical studies.

Non-GAAP selling, general and administrative expenses increased primarily due to higher costs to support Gilead's geographic expansion of its business, partially offset by a decrease in our Branded Prescription Drug fee expense.

Cash, Cash Equivalents and Marketable Securities

As of March 31, 2016, Gilead had $21.3 billion of cash, cash equivalents and marketable securities compared to $26.2 billion as of December 31, 2015. During the first quarter of 2016, we utilized $8.0 billion on stock repurchases and made an upfront license fee payment of $300 million and an equity investment of $425 million related to our license and collaboration agreement with Galapagos NV. Cash flow from operating activities was $3.9 billion for the quarter.

Full Year 2016 Guidance

Gilead reiterates its full year 2016 guidance, initially provided on February 2, 2016:

	Provided
(In millions, except percentages and per share amounts)	
	February 2, 2016
Net Product Sales	$30,000 - $31,000
Non-GAAP*	
Product Gross Margin	88% - 90%
R&D expenses	$3,200 - $3,500
SG&A expenses	$3,300 - $3,600
Effective Tax Rate	18.0% - 20.0%

Corporate Highlights

- Announced that Chairman and Chief Executive Officer (CEO) John C. Martin, PhD assumed the role of Executive Chairman of the company. John F. Milligan, PhD, formerly President and Chief Operating Officer, was promoted to President and CEO, effective March 10, 2016, and appointed to the company's Board of Directors.
- Announced that Gilead will provide grants for up to three years to academic institutions, nonprofit organizations and community groups engaged in HIV cure activities. The unrestricted grants are awarded to organizations with a track record of excellence in results-driven research.
- Announced that the Board of Directors approved the repurchase of an additional $12 billion of the company's common stock which commenced upon completion of the company's existing $15 billion repurchase program authorized in January 2015.

Product & Pipeline Updates announced by Gilead during the First Quarter of 2016 include:

- Announced that U.S. Food and Drug Administration (FDA) approved Odefsey® (emtricitabine 200 mg/rilpivirine 25 mg/tenofovir alafenamide 25 mg or R/F/TAF) for the treatment of HIV-1 infection in certain patients. Emtricitabine and tenofovir alafenamide are from Gilead while rilpivirine is from Janssen Sciences Ireland UC, one of the Janssen Pharmaceutical Companies of Johnson & Johnson. Odefsey is Gilead's second TAF-based regimen to receive FDA approval and represents the smallest pill of any single-tablet regimen available today for the treatment of HIV.
- Announced that the Committee for Medicinal Products for Human Use, the scientific committee of the European Medicines Agency (EMA), adopted a positive opinion on the company's Marketing Authorization Application (MAA) for two doses of Descovy® (emtricitabine 200 mg/tenofovir alafenamide 25 mg, F/TAF), an investigational fixed-dose combination for the treatment of HIV-1 infection in adults and adolescents (ages 12 years and older with body weight at least 35 kg) in combination with other HIV antiretroviral agents.
- Presented data at the 2016 Conference on Retroviruses and Opportunistic Infections, which included the announcement of:
    - 48-week results from a Phase 3 study (Study 1089) evaluating the safety and efficacy of switching virologically suppressed HIV-1 infected adult patients from regimens containing Truvada to regimens containing the investigational fixed-dose combination of emtricitabine and F/TAF. At Week 48, the F/TAF-based regimens were found to be statistically non-inferior to the emtricitabine and tenofovir disoproxil fumarate (F/TDF) -based regimens, based on percentages of patients with HIV-1 RNA levels less than 50 copies/mL. The study also demonstrated statistically significant improvements in renal and bone laboratory parameters among patients receiving F/TAF-based regimens.
    - Results from a preclinical study conducted in collaboration with researchers at Beth Israel Deaconess Medical Center evaluating a proprietary investigational oral toll-like receptor 7 (TLR7) agonist, GS-9620, and a related molecular analogue, GS-986, as part of an HIV eradication strategy. Data from the study conducted in simian immunodeficiency virus (SIV)-infected virally suppressed rhesus macaques on antiretroviral therapy (ART) demonstrate that TLR7 agonist treatment induced transient plasma SIV RNA blips and reduced SIV DNA. In addition, TLR7 agonist treatment resulted in subsequent prolonged virus suppression in some of the macaques after stopping ART.
- Announced that the company's Type II variation application for once-daily Truvada in combination with safer sex practices to reduce the risk of sexually acquired HIV-1 infection among uninfected adults at high risk, a strategy known as pre-exposure prophylaxis or PrEP, was fully validated and under evaluation by the EMA.
- Announced that the company's MAA for TAF 25 mg, an investigational, once-daily treatment for adults with chronic hepatitis B virus (HBV) infection, was fully validated and under assessment by the EMA. The company also submitted a new drug application (NDA) to FDA for TAF 25 mg for the treatment for adults with chronic HBV infection.
- Announced that FDA approved two supplemental indications for Harvoni for use in chronic hepatitis C patients with advanced liver disease. Harvoni in combination with ribavirin for 12 weeks was approved for use in chronic hepatitis C virus (HCV) genotype 1- or 4-infected liver transplant recipients without cirrhosis or

with compensated cirrhosis (Child-Pugh A), and for HCV genotype 1-infected patients with decompensated cirrhosis (Child-Pugh B or C), including those who have undergone liver transplantation. Harvoni is approved for use in HCV genotypes 1, 4, 5 and 6, HCV/HIV-1 coinfection, HCV genotype 1 and 4 liver transplant recipients, and genotype 1-infected patients with decompensated cirrhosis.

- Announced that FDA granted priority review to the company's NDA for an investigational once-daily fixed-dose combination of sofosbuvir and velpatasvir (SOF/VEL), for the treatment of chronic genotype 1-6 HCV infection. FDA has set a target action date under the Prescription Drug User Fee Act of June 28, 2016.

About Gilead

Gilead Sciences is a biopharmaceutical company that discovers, develops and commercializes innovative therapeutics in areas of unmet medical need. The company's mission is to transform and simplify care for people with life-threatening illnesses around the word. Headquartered in Foster City, California, Gilead has operations in North and South America, Europe and Asia-Pacific.

http://www.gilead.com/news/press-releases/2016/4/gilead-sciences-announces-first-quarter-2016-financial-results

Regeneron Pharmaceuticals, Inc. (NASDAQ: REGN)

Regeneron Reports First Quarter 2016 Financial and Operating Results

First quarter 2016 EYLEA® (aflibercept) Injection U.S. net sales increased 44% to $781 million versus first quarter 2015

First quarter 2016 EYLEA global net sales(1) increased 44% to $1.20 billion versus first quarter 2015

Raised estimated full year 2016 EYLEA U.S. net sales growth guidance to 20% - 25% over 2015, from the previous guidance of approximately 20%

Positive dupilumab topline results reported from two Phase 3 trials in atopic dermatitis

TARRYTOWN, N.Y., May 5, 2016 /PRNewswire/ - Regeneron Pharmaceuticals, Inc. (NASDAQ: REGN) today announced financial results for the first quarter of 2016 and provided a business update.

Business Highlights

Marketed Product Update

EYLEA® (aflibercept) Injection for Intravitreal Injection

In the first quarter of 2016, net sales of EYLEA in the United States increased 44% to $781 million from $541 million in the first quarter of 2015. Overall distributor inventory levels remained within the Company's one- to two-week targeted range.

Bayer commercializes EYLEA outside the United States. In the first quarter of 2016, net sales of EYLEA outside of the United States(1) were $419 million, compared to $292 million in the first quarter of 2015. In the first quarter of 2016, Regeneron recognized $146 million from its share of net profit from EYLEA sales outside the United States, compared to $89 million in the first quarter of 2015.

A Phase 3 study of EYLEA for the treatment of non-proliferative diabetic retinopathy in patients without diabetic macular edema (DME) was initiated in the first quarter of 2016.

Praluent® (alirocumab) Injection for the Treatment of High Low-Density Lipoprotein (LDL) Cholesterol

In the first quarter of 2016, net sales of Praluent were $13 million. Product sales for Praluent are recorded by Sanofi, and the Company shares in any profits or losses from the commercialization of Praluent. Praluent was launched in the United States in the third quarter of 2015 and in certain countries in the European Union commencing in the fourth quarter of 2015.

In March 2016, the Company and Sanofi reported data from the Phase 3 ODYSSEY ESCAPE study in patients with heterozygous familial hypercholesterolemia (HeFH) who were undergoing LDL apheresis therapy. The trial achieved its primary endpoint, demonstrating that patients who added Praluent to their existing treatment regimen significantly reduced the frequency of their apheresis therapy by 75%, compared to placebo.

In the first quarter of 2016, the Data Monitoring Committee (DMC) of the ODYSSEY OUTCOMES study for Praluent completed the first interim analysis. In accordance with the protocol, the DMC performed a futility assessment. The DMC recommended the study continue with no changes. Regeneron remains blinded to the actual results of this analysis. The ongoing ODYSSEY OUTCOMES trial is assessing the potential of Praluent to demonstrate cardiovascular benefit.

Pipeline Progress

Regeneron has thirteen product candidates in clinical development. These consist of EYLEA and twelve fully human monoclonal antibodies generated using the Company's VelocImmune® technology, including four in collaboration with Sanofi. In addition to EYLEA and Praluent, highlights from the antibody pipeline include:

Sarilumab, the Company's antibody targeting IL-6R for rheumatoid arthritis, is currently being studied in the global Phase 3 SARIL-RA program.

» In March 2016, the Company and Sanofi reported results from the 24-week Phase 3 SARIL-RA-MONARCH study in adult patients with active rheumatoid arthritis who were inadequate responders to, intolerant of, or inappropriate candidates for methotrexate (MTX) therapy. The study met its primary endpoint, demonstrating that sarilumab monotherapy was superior to adalimumab monotherapy (marketed by AbbVie Inc. as HUMIRA®).

» In December 2015, the U.S. Food and Drug Administration (FDA) accepted for review a Biologics License Application (BLA) for sarilumab, with a target action date of October 30, 2016. Dupilumab, the Company's antibody that blocks signaling of IL-4 and IL-13, is currently being studied in atopic dermatitis, asthma, nasal polyps, and eosinophilic esophagitis.

» In April 2016, the Company and Sanofi reported that the Phase 3 LIBERTY AD SOLO 1 and SOLO 2 trials evaluating dupilumab in adult patients with inadequately controlled moderate-to-severe atopic dermatitis met their primary endpoints.

» A Phase 2 study of dupilumab in pediatric patients (6-17 years of age) with moderate-to-severe atopic dermatitis is fully enrolled and ongoing.

» A Phase 3 pivotal study of dupilumab in patients with uncontrolled persistent asthma continues to enroll patients.

» A Phase 2 study of dupilumab in eosinophilic esophagitis is ongoing.

Fasinumab, the Company's antibody targeting Nerve Growth Factor (NGF), is currently being studied in patients with pain due to osteoarthritis and lower back pain.

» The Company recently reported results from a Phase 2/3 study evaluating fasinumab in patients with moderate-to-severe osteoarthritis pain of the hip or knee who have a history of inadequate pain relief or intolerance to current analgesic therapies. The study met its primary endpoint at 16 weeks.

» In the first quarter of 2016, the Company initiated a Phase 3 long-term safety and efficacy study of fasinumab in patients with pain due to osteoarthritis of the knee or hip, and this trial is currently enrolling patients.

» In the first quarter of 2016, the Company also initiated a Phase 2b/3 study of fasinumab in chronic lower back pain.

REGN2810, an antibody to programmed cell death protein 1 (PD-1), entered a potentially pivotal clinical study for the treatment of advanced cutaneous squamous cell carcinoma in the second quarter of 2016.

Nesvacumab/aflibercept, a combination product comprised of an antibody to angiopoietin-2 (Ang2) co-formulated with aflibercept for intravitreal injection for use in ophthalmology, entered Phase 2 clinical development for the treatment of neovascular age-related macular degeneration (wet AMD) and DME in the first quarter of 2016.

Evinacumab, an antibody to Angptl-3, was granted orphan-drug designation by the FDA in the first quarter of 2016. Clinical studies are ongoing for the treatment of homozygous familial hypercholesterolemia and severe forms of hyperlipidemia.

Human Genetics Initiative

In the first quarter of 2016, the New England Journal of Medicine published a Regeneron Genetics Center paper showing that inactivating mutations of the angiopoeitin-like 4 (ANGPTL4) gene are associated with a significantly reduced risk of coronary artery disease in humans. ANGPTL4 and ANGPTL3 are thought to be related inhibitors of lipoprotein lipase (LPL).

Business Development Update

In March 2016, the Company and Bayer entered into a collaboration agreement to jointly develop a combination therapy of the Ang2 antibody nesvacumab and aflibercept for the treatment of serious eye diseases. Š

In April 2016, the Company and Intellia Therapeutics, Inc. entered into a license and collaboration agreement to advance CRISPR/Cas gene-editing technology for in vivo therapeutic development. In addition to the discovery, development and commercialization of new therapies, the companies will focus on technology development of the CRISPR/Cas platform.

First Quarter 2016 Financial Results

Product Revenues: Net product sales were $784 million in the first quarter of 2016, compared to $545 million in the first quarter of 2015. EYLEA net product sales in the United States were $781 million in the first quarter of 2016, compared to $541 million in the first quarter of 2015.

Total Revenues: Total revenues, which include product revenues described above, increased by 38% to $1.201 billion in the first quarter of 2016, compared to $870 million in the first quarter of 2015. Total revenues also include collaboration revenues of $399 million in the first quarter of 2016, compared to $297 million in the first quarter of 2015. Collaboration revenues in the first quarter of 2016 increased primarily due to higher reimbursement of the Company's research and development expenses under its antibody collaboration with Sanofi, an increase in the Company's net profit from commercialization of EYLEA outside the United States, and reimbursement of the Company's research and development expenses and amortization of up-front payments received in connection with the Company's July 2015 immuno-oncology collaboration with Sanofi.

Refer to Table 4 for a summary of collaboration revenue.

Research and Development (R&D) Expenses: GAAP R&D expenses were $470 million in the first quarter of 2016, compared to $343 million in the first quarter of 2015. The higher R&D expenses in the first quarter of 2016 were principally due to higher development costs primarily related to dupilumab and fasinumab, and higher headcount to support the Company's increased R&D activities, partly offset by lower development costs primarily related to

Praluent. In addition, in the first quarter of 2016, R&D-related non-cash share-based compensation expense was $78 million, compared to $60 million in the first quarter of 2015.

Selling, General, and Administrative (SG&A) Expenses: GAAP SG&A expenses were $290 million in the first quarter of 2016, compared to $159 million in the first quarter of 2015. The increase was primarily due to higher headcount, and higher commercialization expenses related to EYLEA and Praluent. In addition, in the first quarter of 2016, SG&A-related non-cash share-based compensation expense was $60 million, compared to $42 million in the first quarter of 2015.

Cost of Goods Sold (COGS): GAAP COGS was $79 million in the first quarter of 2016, compared to $43 million in the first quarter of 2015. COGS primarily consists of royalties as well as costs in connection with producing U.S. EYLEA commercial supplies, and various start-up costs in connection with the Company's Limerick, Ireland commercial manufacturing facility. COGS increased principally due to the increase in U.S. EYLEA net product sales, as well as an increase in Limerick start-up costs.

Income Tax Expense: In the first quarter of 2016, GAAP income tax expense was $164 million and the effective tax rate was 49.8%, compared to $201 million and 72.5% in the first quarter of 2015. The effective tax rate for the first quarter of 2016 was negatively impacted, compared to the U.S. federal statutory rate, by losses incurred in foreign jurisdictions with rates lower than the federal statutory rate and the non-tax deductible Branded Prescription Drug Fee, partly offset by the federal tax credit for increased research activities and the domestic manufacturing deduction. The effective tax rate for the first quarter of 2015 was negatively impacted primarily by losses incurred in foreign jurisdictions with rates lower than the federal statutory rate, the non-tax deductible Branded Prescription Drug Fee, and expiration, at the end of 2014, of the federal tax credit for increased research activities.

The non-GAAP income tax adjustment in the first quarter of 2016 is primarily related to the cash taxes the Company expects to be paid or payable in 2016 in connection with the immuno-oncology up-front payment that the Company received in 2015, partly offset by the excess tax benefit associated with stock option exercises. The non-GAAP income tax adjustment in the first quarter of 2015 was primarily related to the Company's tax credit carry-forwards available for tax purposes and excess tax benefits in connection with stock option exercises.

Non-GAAP and GAAP Net Income: The Company reported non-GAAP net income of $293 million, or $2.81 per basic share and $2.57 per diluted share, in the first quarter of 2016, compared to non-GAAP net income of $336 million, or $3.28 per basic share and $2.88 per diluted share, in the first quarter of 2015.

The Company reported GAAP net income of $166 million, or $1.59 per basic share and $1.45 per diluted share, in the first quarter of 2016, compared to GAAP net income of $76 million, or $0.74 per basic share and $0.66 per diluted share, in the first quarter of 2015.

http://investor.regeneron.com/common/download/download.cfm?companyid=REGN&fileid=890090&filekey=E07DADA9-1230-4B82-8E7C-4821DD5B593D&filename=REGN_News_2016_5_5_General_Releases.pdf

Seattle Genetics (NASDAQ: SGEN)

Seattle Genetics Reports Second Quarter 2016 Financial Results

-Second Quarter 2016 Revenues Were $95.4 Million, Including Record $66.2 Million in ADCETRIS® (Brentuximab Vedotin) U.S. and Canada Net Sales-

-Top-Line Data from ADCETRIS Phase 3 ALCANZA Trial Expected in Third Quarter of 2016-

-Conference Call Today at 4:30 p.m. ET-

BOTHELL, Wash. - (BUSINESS WIRE) - Jul. 26, 2016 - Seattle Genetics, Inc. (NASDAQ: SGEN) today reported financial results for the second quarter ended June 30, 2016. The company also highlighted ADCETRIS (brentuximab

vedotin) commercialization and clinical development accomplishments, vadastuximab talirine (SGN-CD33A; 33A) activities and progress with its pipeline of antibody-drug conjugates (ADCs) and other proprietary programs.

“We reported record ADCETRIS net sales in the second quarter, which were up 20 percent for the quarter and year-to-date compared to the same periods in 2015. To expand on the ADCETRIS opportunity, we are executing on three ongoing phase 3 clinical trials that are approaching data, starting with ALCANZA top-line results this quarter,” said Clay Siegall, Ph.D., President and Chief Executive Officer of Seattle Genetics. “We also demonstrated progress in the second quarter with our clinical-stage pipeline towards our goal of becoming a multi-product oncology company. We advanced 33A into a phase 3 trial for acute myeloid leukemia (AML) and reported encouraging phase 1 data from two ADCs for urothelial cancer, ASG-15ME and enfortumab vedotin (ASG-22ME). We anticipate advancing several new programs and generating additional data from our pipeline over the remainder of 2016.”

Recent ADCETRIS Highlights

The European Commission approved ADCETRIS for the treatment of adult patients with CD30+ Hodgkin lymphoma at increased risk of relapse or progression following autologous stem cell transplant based on data from the phase 3 AETHERA clinical trial. This is the third approved indication for ADCETRIS in the European Union.

Announced that final data from the ADCETRIS monotherapy pivotal phase 2 clinical trial in relapsed or refractory classical Hodgkin lymphoma were published in the journal Blood. The manuscript, which summarizes the five-year, end-of-study results, highlights that many patients who achieved a complete remission remained in remission at the time of this final analysis.

Takeda continues to receive additional marketing approvals for ADCETRIS, which is now commercially available in 65 countries worldwide.

Recent Vadastuximab Talirine (SGN-CD33A) Highlights

Initiated the pivotal phase 3 CASCADE clinical trial evaluating 33A in combination with the hypomethylating agents (HMAs) azacitidine or decitabine in approximately 500 older patients with newly diagnosed AML. The trial is designed to determine if the 33A-containing regimen improves overall survival compared to patients receiving HMAs alone.
Reported data from a phase 1 trial of 33A plus HMAs in older, newly diagnosed patients with AML in an oral presentation at the 21st Congress of the European Hematology Association (EHA). The data showed a 76 percent objective response rate, including a 41 percent complete remission rate with manageable tolerability profile. The median overall survival for all patients in the phase 1 trial is interim and expected to evolve. The estimated median overall survival for the first 25 patients enrolled in the study was 12.75 months.
Recent Pipeline and Other Highlights

Reported data at the American Society of Clinical Oncology (ASCO) annual meeting from phase 1 trials of ASG-15ME and enfortumab vedotin in metastatic urothelial cancer, primarily bladder carcinoma. The data showed that both ADCs had manageable safety profiles and objective response rates of 40 to 50 percent at the likely recommended doses for future development. ASG-15ME and enfortumab vedotin are being co-developed with Astellas.

Triggered milestones under ongoing ADC collaborations based on progress with programs utilizing Seattle Genetics technology, including from:

Astellas, upon its initiation of a phase 2 clinical trial in metastatic renal cell carcinoma; and,

AbbVie, based on progress with a preclinical program.

Added to and promoted several members of the senior management team, including:

Promoting Naomi Hunder, M.D., to Vice President, Clinical Development. Dr. Hunder joined Seattle Genetics in 2010. She has most recently served as the clinical lead for the ADCETRIS program, notably for the company's successful FDA approval in post-autologous transplant high-risk Hodgkin lymphoma based on data from the phase 3 AETHERA trial.

Promoting Dana Kennedy, Pharm.D., to Vice President, Clinical Development. Dr. Kennedy joined Seattle Genetics in 2007. Her contributions have included the clinical development work that led to the approval of ADCETRIS in systemic anaplastic large cell lymphoma and serving as clinical and program leader for SGN-CD33A.

Hiring Ian Pyrah, Ph.D., as Vice President, Non-Clinical Sciences. Prior to joining Seattle Genetics, Dr. Pyrah spent 10 years at Amgen in several leadership roles, including responsibility for the non-clinical component of a number of successful regulatory submissions.

Hiring Venkat Ramanan, Ph.D., as Vice President, Finance. Dr. Ramanan previously spent nine years at Gilead Sciences and prior to that he was at Amgen and ZS Associates. He has served in a range of roles in finance, business planning and operations supporting U.S. and international markets.

Anticipated ADCETRIS Upcoming Activities

Report top-line data in the third quarter of 2016 from the phase 3 ALCANZA trial in patients with CD30-expressing cutaneous T-cell lymphoma (CTCL).

Report data in the 2017 through mid-2018 timeframe from the phase 3 ECHELON-1 trial in frontline classical Hodgkin lymphoma.

Complete enrollment in the phase 3 ECHELON-2 trial in frontline mature T-cell lymphoma (MTCL) during 2016 and report data in the 2017 to 2018 timeframe.

ADCETRIS is not currently approved for use in CTCL, frontline Hodgkin lymphoma or frontline MTCL.

Anticipated Vadastuximab Talirine (SGN-CD33A) Upcoming Activities

Continue clinical site initiations and enrollment of 500 patients to the pivotal phase 3 CASCADE clinical trial evaluating 33A in combination with HMAs in older patients with newly diagnosed AML.

Report data from ongoing phase 1 trials, including a phase 1b trial of 33A in combination with cytarabine and daunorubicin (7+3) for frontline, younger AML patients.

More information about 33A and ongoing clinical trials can be found at www.ADC-CD33.com.

Anticipated Pipeline Programs Upcoming Activities

Initiate a randomized phase 2 trial of denintuzumab mafodotin (SGN-CD19A; 19A) in frontline diffuse large B-cell lymphoma (DLBCL) during 2016.

Report additional data from phase 1 trials of ASG-15ME and enfortumab vedotin at the European Society for Medical Oncology (ESMO) annual congress being held October 7 to 11, 2016 in Copenhagen, Denmark.

Report clinical data during 2016 from other pipeline programs, including SGN-LIV1A.

Initiate a phase 1 trial of SGN-CD123A in relapsed or refractory AML. SGN-CD123A is an ADC targeted to CD123 utilizing Seattle Genetics' newest technology, comprising an engineered cysteine antibody (EC-mAb) stably linked to a highly potent DNA binding agent called a pyrrolobenzodiazepine (PBD) dimer. CD123 is expressed across AML subtypes, and is particularly prominent on leukemic stem cells.

Advance SGN-CD352A, a novel ADC for multiple myeloma, into a phase 1 clinical trial. SGN-CD352A targets CD352, and utilizes the company's PBD and EC-mAb technology. CD352 is highly expressed on multiple myeloma as well as B-cell malignancies, including chronic lymphocytic leukemia and non-Hodgkin lymphoma.

Second Quarter and Six Months 2016 Financial Results

Total revenues in the quarter and six month periods ended June 30, 2016 increased to $95.4 million and $206.6 million, respectively, compared to $77.1 million and $159.3 million from the same periods in 2015. Revenues included:

ADCETRIS net sales in the second quarter were $66.2 million, a 20 percent increase from net sales of $55.1 million in the second quarter of 2015. For the year-to-date, ADCETRIS sales were $124.9 million, compared to $104.0 million for the year-to-date period in 2015, a 20 percent increase.

Royalty revenues in the second quarter of 2016 were $9.2 million, compared to $7.6 million in the second quarter of 2015. For the year-to-date in 2016, royalty revenues were $41.5 million, compared to $18.7 million for the first six months of 2015. Royalty revenues are primarily driven by international sales of ADCETRIS by Takeda. Royalty revenues for the year-to-date in 2016 also included a $20.0 million sales milestone payment from Takeda earned in the first quarter of 2016.

Amounts earned under the company's ADCETRIS and ADC collaborations totaled $20.0 million in the second quarter and $40.2 million for the first six months of 2016, compared to $14.4 million and $36.6 million for the same periods in 2015.

Total costs and expenses for the second quarter of 2016 were $128.8 million, compared to $124.7 million for the second quarter of 2015. For the first six months of 2016, total costs and expenses were $261.0 million, compared to $228.6 million in the first six months of 2015. The increase in 2016 costs and expenses was primarily driven by progress with ADCETRIS, 33A clinical development and manufacturing activities and investment in the company's pipeline programs.

Non-cash, share-based compensation cost for the first six months of 2016 was $24.3 million, compared to $17.6 million for the same period in 2015.

Net loss for the second quarter of 2016 was $32.7 million, or $0.23 per share, compared to a net loss of $47.5 million, or $0.38 per share, for the second quarter of 2015. For the six months ended June 30, 2016, net loss was $53.2 million, or $0.38 per share, compared to a net loss of $69.2 million, or $0.55 per share, for the same period in 2015.

As of June 30, 2016, Seattle Genetics had $659.5 million in cash, cash equivalents and investments, compared to $712.7 million as of December 31, 2015.

About Seattle Genetics

Seattle Genetics is a biotechnology company focused on the development and commercialization of innovative antibody-based therapies for the treatment of cancer. Seattle Genetics is leading the field in developing antibody-drug conjugates (ADCs), a technology designed to harness the targeting ability of antibodies to deliver cell-killing agents directly to cancer cells. The company's lead product, ADCETRIS® (brentuximab vedotin) is an ADC that, in collaboration with Takeda Pharmaceutical Company Limited, is commercially available for two indications in 50 countries, including the U.S., Canada, Japan and members of the European Union. Additionally, ADCETRIS is being evaluated broadly in more than 30 ongoing clinical trials. Seattle Genetics is also advancing a robust pipeline of clinical-stage programs, including SGN-CD19A, SGN-CD33A, SGN-LIV1A, SGN-CD70A, ASG-22ME and ASG-15ME. Seattle Genetics has collaborations for its ADC technology with a number of leading biotechnology and pharmaceutical companies, including AbbVie, Agensys (an affiliate of Astellas), Bayer, Genentech, GlaxoSmithKline and Pfizer. More information can be found at www.seattlegenetics.com.

Certain of the statements made in this press release are forward looking, such as those, among others, relating to the company's expectations for initiation of future clinical trials, data availability from ongoing clinical trials, expectations for additional regulatory approvals and expectations for financial results for the year 2015. Actual results or developments may differ materially from those projected or implied in these forward-looking statements. Factors that may cause such a difference include that sales of ADCETRIS, royalties and milestone payments and other sources of revenue and our costs and expenses may not be as we expect. We may also be delayed in our planned trial initiations, the conduct of our clinical trials, regulatory submissions and approvals for a variety of reasons. More information about the risks and uncertainties faced by Seattle Genetics is contained in the company's Quarterly Report on Form 10-Q for the quarter ended September 30, 2014 filed with the Securities and Exchange Commission. Seattle Genetics disclaims any intention or obligation to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

http://investor.seattlegenetics.com/phoenix.zhtml?c=124860&p=irol-newsArticle&ID=2188443

Shire Pharmaceuticals (NASDAQ: SHPG)

Shire delivers strong Q1 2016 results with double-digit growth in revenue and Non GAAP earnings per ADS

Proposed combination with Baxalta on track with shareholder votes set for May 27 and closing anticipated in early June 2016

April 29, 2016 – Shire plc ("Shire") (LSE: SHP, NASDAQ: SHPG) announces unaudited results for the three months ended March 31, 2016.

Financial Highlights	Q1 2016	Growth[1]	Non GAAP CER[1][2]
Product sales	$1,627 million	+14%	+16%
Total revenues	$1,709 million	+15%	+17%
Non GAAP operating income	$797 million	+17%	+16%
US GAAP operating income from continuing operations	$544 million	+15%	
Non GAAP EBITDA margin (excluding royalties & other revenues)[3]	46%	0pps[4]	
US GAAP net income margin[5]	25%	-3pps	
Non GAAP net income	$632 million	+13%	
US GAAP net income	$419 million	+2%	
Non GAAP diluted earnings per ADS	$3.19	+12%	+12%
US GAAP diluted earnings per ADS	$2.12	+2%	
Non GAAP cash generation	$492 million	-5%	
Non GAAP free cash flow	$338 million	+38%	
US GAAP net cash provided by operating activities	$390 million	-31%	

[1] Percentages compare to equivalent 2015 period.
[2] On a Constant Exchange Rate ("CER") basis, which is a Non GAAP measure.
[3] Non GAAP earnings before interest, tax, depreciation and amortization ("EBITDA") as a percentage of product sales, excluding royalties and other revenues.

(4) Percentage point change ("pps").
(5) US GAAP net income as a percentage of total revenues.

The Non GAAP financial measures included within this release are explained on pages 25 - 26, and are reconciled to the most directly comparable financial measures prepared in accordance with US GAAP on pages 19 - 22.

First Quarter & Recent Highlights:

- Product sales growth of 14% (16% on a Non GAAP CER basis) to $1.6 billion, driven by VYVANSE®, LIALDA®/MEZAVANT®, CINRYZE®, FIRAZYR®, GATTEX®/REVESTIVE® and NATPARA®.
- Rare disease products acquired from NPS Pharmaceuticals, Inc. ("NPS") continued to perform well with GATTEX/REVESTIVE sales up 247% (up 97% on a pro-forma basis(1)) to $52 million, and NATPARA sales of $16 million.
- Free cash flow remained strong, impacted primarily by net payments and receipts of taxes between Q1 2015 and Q1 2016.
- Lifitegrast New Drug Application ("NDA") accepted by the US Food and Drug Administration ("FDA"), with Prescription Drug User Fee Act ("PDUFA") date set for July 22, 2016.
- Pipeline progression with positive topline results from SHP465 safety and efficacy study in children and adolescents with Attention Deficit Hyperactivity Disorder ("ADHD").
- Completed acquisition of Dyax Corp. ("Dyax") and enrollment on track for SHP643 (formerly DX2930) Phase 3 studies for the treatment of Hereditary Angioedema ("HAE").
- Patent upheld for LIALDA (mesalamine) delayed release tablets by U.S. District Court for the Southern District of Florida; the case has been appealed.
- Baxalta Incorporated ("Baxalta") acquisition on track with integration progressing well; shareholder votes set for May 27 and closing anticipated in early June.

(1) Sales prior to February 21, 2015 were recorded by NPS.

Flemming Ornskov, M.D. Chief Executive Officer, commented:

"Shire is off to a strong start in 2016, delivering double-digit product sales and Non GAAP earnings per ADS growth, and advancing our innovative pipeline. We were pleased to report positive Phase 3 topline results for SHP465 in children and adolescents with ADHD, a therapeutic area with significant need for additional treatment options. We are also looking forward to hearing from the FDA by late July regarding lifitegrast, a potential new treatment for dry eye disease.

While we maintain our sharp focus on Shire's business, we closed the acquisition of Dyax during the quarter and we are making excellent progress with the Baxalta integration planning. Our shareholder vote is scheduled for May 27 and the closing is anticipated to follow in early June. We look forward to officially welcoming our Baxalta colleagues to Shire, and creating a global biotechnology leader focused on rare diseases and other highly specialized conditions."

https://www.shire.com/newsroom/2016/april/shire-delivers-strong-q1-2016-results-with-double-digit-growth

Vertex Pharmaceuticals (NASDAQ: VRTX)

Vertex Reports Second Quarter 2016 Financial Results – 27/7/2016

-Second quarter 2016 cystic fibrosis product revenues of $426 million; $245 million for ORKAMBI® (lumacaftor/ivacaftor) and $180 million for KALYDECO® (ivacaftor)-

-Vertex reiterates 2016 guidance for ORKAMBI product revenues of $1.0 to $1.1 billion and KALYDECO product revenues of $685 to $705 million-

-Pipeline of investigational CF medicines continues to progress and expand with addition of recent Moderna mRNA collaboration-

BOSTON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the quarter ended June 30, 2016 and reviewed recent progress with its approved and investigational cystic fibrosis (CF) medicines. Vertex also reiterated its financial guidance for total 2016 ORKAMBI® and KALYDECO® revenues and expenses. Key financial results include:

	Three Months Ended June 30,		
	2016	2015	% Change
	(in millions, except per share and percentage data)		
ORKAMBI product revenues, net	$ 245	$ —	N/A
KALYDECO product revenues, net	$ 180	$ 155	16%
TOTAL CF product revenues, net	$ 426	$ 155	175%
GAAP net loss	$ (65)	$ (189)	(66)%
GAAP net loss per share	$ (0.26)	$ (0.78)	(67)%
Non-GAAP net income (loss)	$ 58	$ (131)	N/A
Non-GAAP net income (loss) per share	$ 0.24	$ (0.54)	N/A

"Just over a year ago, we received FDA approval for ORKAMBI, marking the most significant step to date in our journey to develop new medicines for potentially all people with CF," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "Today, approximately 27,000 people are eligible for a medicine to treat the cause of their CF, and we're making significant progress toward bringing ORKAMBI and KALYDECO to even more patients while also advancing our pipeline of other potential medicines to enhance the future treatment of CF."

Vertex today reviewed recent progress from across its CF program:

ORKAMBI

Supplemental New Drug Application for the treatment of children ages 6 to 11 accepted for Priority Review by the U.S. FDA: In late May 2016, the U.S. Food and Drug Administration (FDA) granted Vertex's request for Priority Review of a supplemental New Drug Application (sNDA) for approval of ORKAMBI for children ages 6 through 11 who have two copies of the F508del mutation. The FDA set a target review date of September 30, 2016 for a decision on the sNDA. There are approximately 2,400 children ages 6 through 11 who have two copies of the F508del mutation in the U.S.The sNDA was based on data from an open label Phase 3 safety study of ORKAMBI. Data from this study were presented at the 39th European Cystic Fibrosis Society (ECFS) conference on June 10, 2016.

Enrollment complete in Phase 3 study in children ages 6 to 11 to support approval in Europe: Vertex has completed enrollment in a six-month Phase 3 efficacy study evaluating ORKAMBI in children ages 6 through 11 who have two copies of the F508del mutation. The primary endpoint is the absolute change in lung clearance index. Pending data from the study, Vertex plans to submit a Marketing Authorization Application variation in theEuropean Union in the first half of 2017. In Europe, there are approximately 3,400 children ages 6 through 11 who have two copies of the F508del mutation.

Initiation of Phase 3 study of ORKAMBI in children ages 2 to 5: Vertex recently initiated a Phase 3 study of ORKAMBI in children ages 2 to 5. Similar to the study of KALYDECO in children in this age group, the first part of the two-part study is evaluating pharmacokinetics and safety to inform dose selection for the second part of the study. The primary endpoint of the second part of the study is safety and tolerability, with multiple efficacy measurements as secondary endpoints.

KALYDECO

Regulatory filing for patients with residual function mutations: In October 2015, Vertex submitted an sNDA for approval of KALYDECO for treatment of people with CF ages 2 and older who have one of 23 residual function mutations and received a Complete Response Letter on this sNDA inFebruary 2016. There are approximately 1,500 people ages 2 and older in the U.S. who have one of the 23 residual function mutations included in the sNDA, and Vertex continues to pursue FDA approval of KALYDECO for these patients as soon as possible.

VX-661 in Combination with Ivacaftor

Data from Phase 3 study in people with two copies of F508del mutation expected in first half of 2017: Vertex today announced that it expects to complete enrollment of a 24-week Phase 3 placebo-controlled study evaluating the investigational combination of VX-661 and ivacaftor in people ages 12 and older who have two copies of the F508del mutation in August 2016. Data from this study are expected in the first half of 2017. The remaining three Phase 3 studies of VX-661 in combination with ivacaftor are proceeding as outlined in the company's April 27, 2016 press release. Vertex plans to submit a New Drug Application (NDA) to the FDA for VX-661 in combination with ivacaftor in the second half of 2017, pending data from the Phase 3 program.

Next-Generation Correctors

Ongoing Phase 1 studies in healthy volunteers: Vertex's two next-generation correctors known as VX-152 and VX-440 are being evaluated alone and as part of a triple combination with VX-661 and ivacaftor in ongoing Phase 1 studies in healthy volunteers. Pending data from the Phase 1 studies, the company expects to begin Phase 2 clinical development in people with CF to evaluate one or both of the next-generation correctors with VX-661 and ivacaftor in the second half of 2016.

New Collaboration to Advance Future Treatment of CF

Collaboration with Moderna Therapeutics focused on mRNA Therapeutics for CF: In early July, Vertex entered into an exclusive research collaboration and licensing agreement with Moderna Therapeutics aimed at the discovery and development of messenger Ribosomal Nucleic Acid (mRNA) therapies for the treatment of CF. The collaboration will focus on the use of mRNA therapies to treat the underlying cause of CF by enabling cells in the lungs to produce functional copies of the cystic fibrosis transmembrane conductance regulator (CFTR) protein, which is known to be defective in people with CF. As part of the collaboration, Vertex made an up-front payment of $20 million to Moderna as well as a $20 million equity investment. The investment will provide Vertex with an ownership stake in Moderna. Vertex will also pay Moderna future development and regulatory milestones of up to$275 million, including $220 million in approval and reimbursement milestones, as well as tiered royalty payments on future sales.
Second Quarter 2016 Financial Highlights

Revenues:

- Net product revenues from ORKAMBI were $245.5 million. ORKAMBI was launched in the U.S. in July 2015.
- Net product revenues from KALYDECO were $180.2 million, compared to $154.9 million for the second quarter of 2015.

Expenses:

- GAAP operating expenses were $428.3 million compared to $337.2 million for the second quarter of 2015. Non-GAAP operating expenses (combined non-GAAP R&D and SG&A) were $306.3 million compared to $253.9 million for the second quarter of 2015. The increases were primarily driven by increased costs related to the progression of our CF pipeline and to increased investment in global commercial support for the launch of ORKAMBI.
- GAAP R&D expenses were $271.0 million compared to $223.9 million for the second quarter of 2015. Non-GAAP R&D expenses were $217.7 millioncompared to $181.9 million for the second quarter of 2015. The increases were primarily driven by increased investment to progress our portfolio of CF medicines.
- GAAP SG&A expenses were $111.7 million compared to $94.4 million for the second quarter of 2015. Non-GAAP SG&A expenses were $88.6 millioncompared to $72.0 million for the second quarter of 2015. The increases were primarily driven by increased investment to support the global launch of ORKAMBI.

Net Income (Loss) Attributable to Vertex:

- GAAP net loss was $(64.5) million, or $(0.26) per diluted share, compared to GAAP net loss of $(188.8) million, or $(0.78) per diluted share, for the second quarter of 2015. Non-GAAP net income was $58.0 million, or $0.24 per diluted share, compared to a non-GAAP net loss of $(130.7) million, or$(0.54) per diluted share, for the second quarter of 2015.

Cash Position:

- As of June 30, 2016, Vertex had $1.07 billion in cash, cash equivalents and marketable securities compared to $1.04 billion in cash, cash equivalents and marketable securities as of December 31, 2015.
- As of June 30, 2016, Vertex had $300 million outstanding from a credit agreement, repayable by the end of the third quarter of 2017.

2016 Financial Guidance:

Vertex today reiterated its 2016 revenue guidance for ORKAMBI and KALYDECO. The company also reiterated guidance for its 2016 combined non-GAAP R&D and SG&A expenses. The guidance is summarized below:

- ORKAMBI: The company continues to expect total 2016 product revenues for ORKAMBI of $1.0 to $1.1 billion. As of June 30, 2016, approximately 6,000 patients had initiated treatment with ORKAMBI in the U.S. In addition to revenues from the use of ORKAMBI in patients ages 12 and older in theU.S., the 2016 ORKAMBI guidance also reflects potential revenues from the anticipated use of ORKAMBI in the U.S. for the treatment of people ages 6 to 11 who have two copies of the F508del mutation in the fourth quarter of 2016, pending FDA approval, and revenues from sales of ORKAMBI outside the U.S., primarily in Germany.
- KALYDECO: The company continues to expect total 2016 product revenues for KALYDECO of $685 to $705 million. 2016 guidance for KALYDECO currently excludes any revenues related to the potential approval of KALYDECO for people in the U.S. who have residual function mutations.
- Operating Expenses (Combined Non-GAAP R&D and SG&A Expenses): Vertex continues to expect that its combined non-GAAP R&D and SG&A expenses in 2016 will be in the range of $1.18 to $1.23 billion.

Vertex's expected non-GAAP R&D and SG&A expenses exclude stock-based compensation expense and certain other expenses.

About Vertex

Vertex is a global biotechnology company that aims to discover, develop and commercialize innovative medicines so people with serious diseases can lead better lives. In addition to our clinical development programs focused on cystic fibrosis, Vertex has more than a dozen ongoing research programs aimed at other serious and life-threatening diseases.

Founded in 1989 in Cambridge, Mass., Vertex today has research and development sites and commercial offices in the United States, Europe, Canada and Australia. For six years in a row, Science magazine has named Vertex one of its Top Employers in the life sciences. For additional information and the latest updates from the company, please visit www.vrtx.com.

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