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

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**FORM 8-K**

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**CURRENT REPORT**

**PURSUANT TO SECTION 13 OR 15(D) OF THE  
SECURITIES EXCHANGE ACT OF 1934**

**Date of report (Date of earliest event reported): October 20, 2010**

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**ALEXION PHARMACEUTICALS, INC.**

(Exact name of registrant as specified in its charter)

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

**000-27756**  
(Commission  
File Number)

**13-3648318**  
(I.R.S. Employer  
Identification No.)

**352 Knottter Drive, Cheshire, Connecticut 06410**  
(Address of Principal Executive Offices) (Zip Code)

**Registrant's telephone number, including area code: (203) 272-2596**

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Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
  - Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
  - Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
  - Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))
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**Item 2.02 Results of Operations and Financial Condition.**

On October 21, 2010, Alexion issued a press release relating to its results of operations and financial conditions for the quarter ended September 30, 2010. A copy of the press release is furnished as Exhibit 99.1 to this Form 8-K.

The attached press release contains both U.S. Generally Accepted Accounting Principles (“GAAP”) and non-GAAP financial measures. The non-GAAP financial measures exclude share-based compensation expenses and taxes not payable in cash. Reconciliations between non-GAAP and GAAP financial measures are included in the press release set forth as Exhibit 99.1 furnished to this Form 8-K. The Company’s management utilizes non-GAAP financial information to provide a useful measure of comparative operating performance of the Company. The non-GAAP financial measures are supplemental to and not a substitute for, measures of financial performance prepared in accordance with GAAP.

**Item 8.01 Other Events.**

On October 20, 2010, Alexion announced that its two open-label Phase 2 studies investigating eculizumab (Soliris<sup>®</sup>) as a treatment for patients with atypical Hemolytic Uremic Syndrome met the primary and key secondary endpoints with high clinical and statistical significance, in interim analyses. A copy of the press release is filed as Exhibit 99.2 to this Form 8-K.

**Item 9.01 Financial Statements and Exhibits.**

(d) Exhibits

99.1 Press Release issued by Alexion Pharmaceuticals, Inc. on October 21, 2010 relating to its results of operations and financial conditions for the quarter ended September 30, 2010.

99.2 Press Release issued by Alexion Pharmaceuticals, Inc. on October 20, 2010 relating to its two Phase 2 studies investigating eculizumab as a treatment for patients with atypical Hemolytic Uremic Syndrome.

**Signature**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

ALEXION PHARMACEUTICALS, INC.

Date: October 21, 2010

By: /s/ Thomas I.H. Dubin

Name: Thomas I. H. Dubin

Title: Senior Vice President and Chief Legal Officer

**Contacts:**

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**ALEXION REPORTS THIRD QUARTER 2010 RESULTS**

*- Strong initial launch of Soliris® in Japan -*

*- Steady addition of new patients on Soliris in U.S. and Europe -*

*- Guidance raised for 2010 revenues and non-GAAP EPS -*

*- Positive interim data from current studies of eculizumab in patients with aHUS -*

**Third Quarter 2010 — Selected Highlights:****Financial:**

- Soliris® (eculizumab) net product sales increased 38 percent to \$141.6 million in Q3 2010, compared to \$102.6 million in Q3 2009.
- Q3 GAAP net income increased to \$27.9 million, or \$0.30 per share, compared to GAAP net income of \$26.7 million, or \$0.29 per share, in Q3 2009. (Q3 2009 GAAP net income benefited from a tax rate of 3.4 percent, since it was prior to the Company's Q4 2009 valuation allowance reversal.)
- Q3 non-GAAP net income increased 40 percent to \$47.2 million, or \$0.50 per share, compared to non-GAAP net income of \$33.7 million, or \$0.37 per share, in Q3 2009.

**Clinical Development:**

- Interim results from Phase 2 studies of eculizumab in patients with atypical Hemolytic Uremic Syndrome (aHUS) published in ASN abstracts
- Study of eculizumab in children with aHUS commenced
- Transplant programs progress

**Cheshire, CT, October 21, 2010** - Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) today announced financial results for the three and nine months ended September 30, 2010.

**Third Quarter 2010 Financial Results:**

For the three months ended September 30, 2010, Alexion Pharmaceuticals, Inc. ("Alexion" or the "Company") reported total revenues of \$141.6 million from net product sales of Soliris® (eculizumab), compared to \$102.6 million in Q3 2009, reflecting the strong addition of new patients in the U.S. and in European countries, and the initial launch of Soliris in Japan, during the quarter.

Soliris is the only treatment specifically indicated for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH), an ultra-rare, debilitating and life-threatening blood disorder. Soliris was approved by the U.S. Food and Drug Administration and the European Commission in 2007 and by Japan's Ministry of Health, Labour and Welfare in 2010.

Alexion's non-GAAP operating results are equal to its GAAP operating results adjusted for only share-based compensation and non-cash tax expense. Non-cash tax expense represents the reduction in cash taxes attributable to the utilization of U.S. net operating losses. The following summary table is provided for investors' convenience.

(in thousands, except per share data)  
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2010	2009	2010	2009
Total revenues	\$ 141,569	\$ 102,628	\$ 384,982	\$ 276,151
GAAP net income	\$ 27,873	\$ 26,731	\$ 70,580	\$ 58,039
Share-based compensation	8,379	6,979	24,733	21,853
Non-cash tax expense	10,931	—	23,369	—
Non-GAAP net income	\$ 47,183	\$ 33,710	\$ 118,682	\$ 79,892
Shares used in computing diluted earnings per share (GAAP)	93,021	90,946	92,580	90,246
Shares used in computing diluted earnings per share (non-GAAP)	94,217	92,143	93,823	91,488
GAAP earnings per share - diluted	\$ 0.30	\$ 0.29	\$ 0.76	\$ 0.65
Non-GAAP earnings per share - diluted	\$ 0.50	\$ 0.37	\$ 1.27	\$ 0.88

### Third Quarter 2010 Non-GAAP Financial Results:

The Company reported non-GAAP net income for Q3 2010 of \$47.2 million, or \$0.50 per share, an increase of 40 percent compared to \$33.7 million, or \$0.37 per share, in Q3 2009.

Alexion's non-GAAP operating expenses for Q3 2010 were \$74.3 million, compared to \$55.9 million for Q3 2009. Non-GAAP research and development (R&D) expenses for Q3 2010 were \$23.1 million, compared to \$19.2 million for Q3 2009. The increase in R&D expenses primarily reflected the Company's expanded clinical development programs. Non-GAAP selling, general and administrative (SG&A) expenses for Q3 2010 were \$51.1 million, compared to \$36.7 million for Q3 2009. The increase in SG&A expenses primarily reflected costs associated with the expansion of the Company's worldwide operations.

**Third Quarter 2010 GAAP Financial Results:**

The Company reported GAAP net income for Q3 2010 of \$27.9 million, or \$0.30 per share, compared to \$26.7 million, or \$0.29 per share, in Q3 2009. Q3 2009 GAAP net income benefited from a tax rate of 3.4 percent, since it was prior to the reversal of the valuation allowance on U.S. deferred tax assets. Alexion's GAAP operating expenses for Q3 2010 were \$82.4 million, compared to \$62.8 million for Q3 2009. GAAP R&D expenses for Q3 2010 were \$25.2 million, compared to \$21.3 million for Q3 2009. GAAP SG&A expenses were \$57.2 million for Q3 2010, compared to \$41.5 million for Q3 2009.

As of September 30, 2010, the Company had \$298.6 million in cash, cash equivalents and marketable securities, compared to \$248.8 million at June 30, 2010.

"We are pleased with the continued growing strength of our global operations in the third quarter, highlighted by the success of the initial launch of Soliris for patients with PNH in Japan," said Leonard Bell, M.D., Chief Executive Officer. "Further, we are encouraged by the positive interim data from our two studies of eculizumab in patients with aHUS and look forward to advancing this important program."

**2010 Financial Guidance:**

The Company is raising its 2010 financial guidance for revenues and non-GAAP earnings per share (EPS). Guidance for 2010 worldwide net product sales has been raised from the previously provided range of \$515 to \$530 million to the higher range of \$536 to \$538 million. Guidance for 2010 non-GAAP EPS has been raised from the previous range of \$1.63 to \$1.68 to the higher range of \$1.73 to \$1.75, based on a forecast of approximately 94 million fully diluted shares outstanding for 2010.

Guidance for 2010 non-GAAP operating expenses is being narrowed within the upper end of the previously provided guidance range, now to \$290 to \$295 million. Within this 2010 non-GAAP operating expense guidance, R&D expense guidance has been lowered from the previous range of \$95 to \$100 million to the lower range of \$92 to \$94 million, while 2010 guidance for non-GAAP SG&A expenses has been raised from the previous range of \$190 to \$195 million to the higher range of \$198 to \$201 million. The increase in non-GAAP SG&A expense guidance relates primarily to accelerated investment in the Company's nephrology therapeutic area and the earlier than expected launch of Soliris in Japan. The Company is raising its guidance for its 2010 GAAP tax rate from a previous range of 30 to 32 percent to the higher range of 32 to 33 percent. The Company is lowering its guidance for its 2010 non-GAAP tax rate, which excludes non-cash tax expense, from a previous range of 11 to 12 percent to the lower range of 9 to 11 percent.

The Company is reiterating other elements of its 2010 fiscal year guidance. Cost of sales is anticipated to be in the range of 12 to 13 percent, and share-based compensation expense for the year is expected to be in a range of \$32 to \$34 million.

**Third Quarter 2010 Research and Development Progress:**

During the third quarter, Alexion made continued progress on advancing the development of Soliris as a treatment for patients suffering from ultra-rare and severe complement-mediated disorders beyond PNH, with a focus on its two lead nephrology programs: aHUS and transplant.

## **Atypical Hemolytic Uremic Syndrome (aHUS)**

### Interim Data from Current Studies

Alexion has announced that, in interim analyses, its two open-label Phase 2 studies investigating eculizumab as a treatment for patients with atypical Hemolytic Uremic Syndrome (aHUS) have met the primary and key secondary endpoints with high clinical and statistical significance.

These two clinical studies investigate eculizumab for the treatment of patients with aHUS who (i) were resistant or intolerant to plasma therapy, or (ii) were receiving plasma therapy chronically. The studies include adolescent and adult patients and are ongoing. In both studies, interim results were reported at the last captured time point. Eculizumab appeared to be well-tolerated in the studies, with the most common adverse events including anemia, diarrhea, headache, nausea and hypertension.

aHUS is an ultra-rare, chronic and life-threatening disease in which uncontrolled complement activation causes blood clots in small blood vessels (thrombotic microangiopathy, or TMA) throughout the body leading to stroke, heart attack, kidney failure and death.<sup>1,2</sup> Approximately 60 percent of patients with aHUS require dialysis, a kidney transplant, or die within a year of diagnosis.<sup>2</sup> Abstracts summarizing these interim data have been posted on the web site of the American Society of Nephrology (ASN) at <http://www.abstracts2view.com/asn/>. These two trials are currently ongoing, and data are expected to be presented at the ASN annual meeting held November 18 to 21 in Denver, Colorado.

### Pediatric Study

Alexion has commenced a Phase 2, open-label, single-arm, multi-center study of eculizumab in pediatric patients with aHUS in the United States, European Union and Canada. Information about the trial is posted on [www.clinicaltrials.gov](http://www.clinicaltrials.gov), Identifier Number NCT01193348. Physicians and families who are interested in participating in this clinical trial can learn more by contacting Alexion by e-mail at [clinicaltrials@alxn.com](mailto:clinicaltrials@alxn.com), or by visiting the Alexion website at [www.alexionpharma.com](http://www.alexionpharma.com) and clicking on the clinical trials link.

## **Transplant: Acute Humoral Rejection (AHR)**

Eculizumab is being investigated as a treatment for patients undergoing kidney transplant who are at elevated risk of antibody mediated rejection, also known as acute humoral rejection, or AHR. The Company is supporting investigator-initiated studies in elevated-risk kidney transplantation in the U.S. and Australia. Separately, an investigator-initiated study in patients with ABO blood-type incompatibility is enrolling. Alexion is now planning a single, global, company-sponsored controlled clinical trial using eculizumab to prevent AHR in patients undergoing kidney transplant. The global study is expected to commence in multiple centers next year following protocol finalization.

## **Oncology Program: Samalizumab**

As previously announced, Alexion has completed enrollment in its Phase I single dose, dose-escalation clinical study of samalizumab, its anti-CD200 antibody, in patients with treatment refractory chronic lymphocytic leukemia or multiple myeloma. The trial has enrolled 26 patients. Several patients in the trial have elected to receive multiple doses of samalizumab in an extension of the original trial. A further presentation of data from this study is expected before the end of the year.

## **Conference Call/Web Cast Information**

Alexion will host a conference call/webcast to discuss matters mentioned in this release. The call is scheduled for today, October 21, at 10:00 a.m., Eastern Time. To participate in this call, dial 719-457-2631, confirmation code 3490595, shortly before 10:00 a.m., Eastern Time. A replay of the call will be available for a limited period following the call, beginning at 2:00 p.m., Eastern Time. The replay number is 719-457-0820, confirmation code 3490595. The audio webcast can be accessed at [www.alexionpharma.com](http://www.alexionpharma.com).

## **About Soliris**

Soliris is a first-in-class terminal complement inhibitor developed from the laboratory through regulatory approval and commercialization by Alexion. Soliris has been approved in the U.S., European Union, Japan and other territories as the first treatment for patients with PNH, an ultra-rare, debilitating and life-threatening blood disorder defined by uncontrolled complement activation which causes chronic hemolysis, or the destruction of red blood cells. Prior to these approvals, there were no therapies specifically available for the treatment of patients with PNH. Eculizumab (Soliris) is not approved for the treatment of aHUS, transplant or other indications other than PNH. Alexion's innovative approach to complement inhibition has received some of the pharmaceutical industry's highest honors: the 2008 Prix Galien USA Award for Best Biotechnology Product with broad implications for future biomedical research and the 2009 Prix Galien France Award in the category of Drugs for Rare Diseases. More information on Soliris is available at [www.soliris.net](http://www.soliris.net).

## **About Alexion**

Alexion Pharmaceuticals, Inc. is a biopharmaceutical company working to develop and deliver life-changing treatments for patients with serious and life-threatening medical conditions. Alexion is engaged in the discovery, development and commercialization of therapeutic products aimed at treating patients with a wide array of severe disease states, including hematologic and kidney diseases, transplant, other inflammatory disorders, and cancer. Soliris is Alexion's first marketed product, and the Company is pursuing potential new indications for Soliris beyond PNH, as well as early-stage development of other antibody product candidates. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at [www.alexionpharma.com](http://www.alexionpharma.com).

This press release includes certain non-GAAP financial amounts that are adjusted from GAAP amounts. Alexion believes that these non-GAAP financial amounts, when considered together with the GAAP amounts, can enhance an overall understanding of Alexion's past financial performance and its prospects for the future. The non-GAAP financial amounts are included with the intent of providing both management and investors with a more complete understanding of underlying operational results and trends. In addition, these non-GAAP financial amounts are among the primary indicators Alexion management uses for planning and forecasting purposes and for measuring the Company's performance.

These non-GAAP financial amounts are not intended to be considered in isolation or as a substitute for GAAP amounts. A reconciliation of GAAP to non-GAAP amounts is included in this press release.



*This news release contains forward-looking statements, including statements related to guidance regarding anticipated financial results for 2010, projected tax rates, assessment of the Company's financial position and commercialization efforts, including with respect to the commercial launch of Soliris in Japan, potential benefits and commercial potential for Soliris, potential of Alexion's complement-inhibition technology for treatment of diseases other than PNH; plans for clinical programs for Soliris in aHUS and other non-PNH indications and for samalizumab; and progress in developing commercial infrastructure. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including for example, decisions of regulatory authorities regarding marketing approval or material limitations on the marketing of Soliris, delays in arranging satisfactory manufacturing capabilities and establishing commercial infrastructure, delays in developing or adverse changes in commercial relationships, the possibility that results of clinical trials are not predictive of safety and efficacy results of Soliris in broader patient populations, the possibility that initial results of commercialization are not predictive of future rates of adoption of Soliris, the risk that third parties will not agree to license any necessary intellectual property to Alexion on reasonable terms or at all, the risk that third party payors (including governmental agencies) will not reimburse for the use of Soliris at acceptable rates or at all, the risk that estimates regarding the number of patients with PNH or other disorders is inaccurate, and a variety of other risks set forth from time to time in Alexion's filings with the Securities and Exchange Commission, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended June 30, 2010 and in Alexion's other filings with the Securities and Exchange Commission. Alexion does not intend to update any of these forward-looking statements to reflect events or circumstances after the date hereof, except when duty arises under law.*

#### References

- (1) Hosler GA, Cusumano AM, Hutchins GM. Thrombotic thrombocytopenic purpura and hemolytic uremic syndrome are distinct pathologic entities. A review of 56 autopsy cases. Arch Pathol Lab Med 2003 Jul;127(7):834-9.
- (2) Loirat C, Noris M, Fremeaux-Bacchi V. Complement and the atypical hemolytic uremic syndrome in children. Pediatr Nephrol. 2008 Nov;23(11):1957-72.

(Tables Follow)

**ALEXION PHARMACEUTICALS, INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS**  
(in thousands, except per share amounts)  
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2010	2009	2010	2009
Net product sales	\$ 141,569	\$ 102,628	\$ 384,982	\$ 276,151
Cost of sales (1)	16,495	11,895	44,215	32,167
Operating expenses:				
Research and development (1)	25,153	21,323	71,217	58,700
Selling, general and administrative (1)	57,208	41,523	163,941	120,880
Total operating expenses	<u>82,361</u>	<u>62,846</u>	<u>235,158</u>	<u>179,580</u>
Operating income	42,713	27,887	105,609	64,404
Other expense	(106)	(205)	(845)	(289)
Debt exchange expense	—	—	—	(3,395)
Income before income taxes	42,607	27,682	104,764	60,720
Income tax provision	14,734	951	34,184	2,681
Net income	<u>\$ 27,873</u>	<u>\$ 26,731</u>	<u>\$ 70,580</u>	<u>\$ 58,039</u>
Earnings per common share				
Basic	<u>\$ 0.31</u>	<u>\$ 0.31</u>	<u>\$ 0.79</u>	<u>\$ 0.69</u>
Diluted	<u>\$ 0.30</u>	<u>\$ 0.29</u>	<u>\$ 0.76</u>	<u>\$ 0.65</u>
Shares used in computing earnings per common share				
Basic	<u>89,490</u>	<u>87,447</u>	<u>89,003</u>	<u>84,464</u>
Diluted	<u>93,021</u>	<u>90,946</u>	<u>92,580</u>	<u>90,246</u>

(1) The following is the share-based compensation expense included in the respective captions of the condensed consolidated statements of operations above:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2010	2009	2010	2009
Share-based compensation expense:				
Cost of sales	\$ 290	\$ —	\$ 855	\$ —
Research and development	2,029	2,108	6,139	6,163
Selling, general and administrative	6,060	4,871	17,739	15,690
	<u>\$ 8,379</u>	<u>\$ 6,979</u>	<u>\$ 24,733</u>	<u>\$ 21,853</u>

**ALEXION PHARMACEUTICALS, INC.**  
**CONDENSED CONSOLIDATED BALANCE SHEETS**  
(in thousands)  
(unaudited)

	<u>September 30,</u> <u>2010</u>	<u>December 31,</u> <u>2009</u>
Cash, cash equivalents and marketable securities	\$ 298,554	\$ 176,220
Trade accounts receivable, net	154,088	113,731
Inventories, net	60,412	40,885
Deferred tax assets, current	19,265	16,726
Other current assets	25,469	25,894
Property, plant and equipment, net	162,111	164,691
Deferred tax assets, noncurrent	164,446	194,308
Other noncurrent assets	57,463	53,946
Total assets	<u>\$ 941,808</u>	<u>\$ 786,401</u>
Accounts payable and accrued expenses	\$ 101,521	\$ 83,187
Other current liabilities	15,693	2,075
Long term debt	3,718	9,918
Other noncurrent liabilities	9,060	2,865
Total liabilities	<u>129,992</u>	<u>98,045</u>
Total stockholders' equity	<u>811,816</u>	<u>688,356</u>
Total liabilities and stockholders' equity	<u>\$ 941,808</u>	<u>\$ 786,401</u>



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## **Alexion Announces Interim Results from Phase 2 Trials of Eculizumab (Soliris®) in Patients with atypical Hemolytic Uremic Syndrome (aHUS)**

### **Data Accepted for Presentation at American Society of Nephrology Annual Meeting in November**

CHESHIRE, Conn.— Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) today announced that its two open-label Phase 2 studies investigating eculizumab (Soliris®) as a treatment for patients with atypical Hemolytic Uremic Syndrome (aHUS) have met the primary and key secondary endpoints with high clinical and statistical significance, in interim analyses. aHUS is an ultra-rare, chronic and life-threatening disease in which uncontrolled complement activation causes blood clots in small blood vessels (thrombotic microangiopathy, or TMA) throughout the body leading to stroke, heart attack, kidney failure and death.<sup>1,2</sup> Approximately 60 percent of patients with aHUS require dialysis, a kidney transplant or die within a year of diagnosis.<sup>2</sup> Abstracts summarizing these interim data have been posted on the web site of the American Society of Nephrology (ASN) at <http://www.abstracts2view.com/asn/>. These two trials are currently ongoing and data will be presented at the ASN annual meeting held November 18-21 in Denver, Colorado.

#### **Patients Resistant to Plasma Therapy**

Abstract 1338, "Safety and Efficacy of Eculizumab in aHUS Patients Resistant to Plasma Therapy: Interim Analysis from a Phase 2 Trial," summarized an interim analysis of 17 adolescent and adult patients with aHUS who were resistant to plasma therapy and were treated with eculizumab for up to 26 weeks.<sup>3</sup> The primary endpoint of the study is the change in platelet count, a measure of TMA. In this interim analysis, researchers observed a significant  $80 \pm 64 \times 10^3/\mu\text{L}$  ( $p < 0.0001$ ) increase in platelet count with eculizumab treatment compared to baseline. Key secondary clinical endpoints were also positive. Updated data from this study will be presented at the ASN annual meeting on Saturday, November 20 at 5:30 p.m. Mountain Standard Time (MST).

## **Patients on Chronic Plasma Therapy**

Abstract #157, “Safety and Efficacy of Eculizumab in aHUS Patients on Chronic Plasma Therapy: Interim Analysis of a Phase 2 Trial,” summarized interim results from a study of 20 adolescent and adult patients with aHUS who were receiving plasma therapy chronically prior to entering the study.<sup>4</sup> The primary endpoint of the study is TMA Event-Free Status, as defined by stable platelet counts, absence of plasma therapy and no new dialysis. In this interim analysis of 15 patients treated with eculizumab for at least 12 weeks, a significant 87% (95% CI 60-98) of patients achieved TMA Event-Free Status. Key secondary clinical endpoints were also positive. Interim data from this study will be presented in a poster session at the ASN annual meeting on Friday, November 19 at 10:00 a.m. MST.

In both studies, interim results were reported at the last captured timepoint. Eculizumab appeared to be well-tolerated in the studies, with the most common adverse events including anemia, diarrhea, headache, nausea and hypertension.

## **About the Studies**

These two open-label Phase 2 clinical studies investigate eculizumab for the treatment of patients with aHUS who (i) were resistant or intolerant to plasma therapy, or (ii) were receiving plasma therapy chronically. The studies include adolescent and adult patients and are ongoing.

## **Pediatric Study**

Alexion has commenced a Phase 2, open-label, single-arm, multi-center study of eculizumab in pediatric patients with aHUS in the United States, European Union and Canada. Information about the trial is posted to [www.clinicaltrials.gov](http://www.clinicaltrials.gov), Identifier Number NCT01193348. Physicians and families who are interested in participating in this clinical trial can learn more by contacting Alexion by e-mail at [clinicaltrials@alxn.com](mailto:clinicaltrials@alxn.com), or by visiting the Alexion website at [www.alexionpharma.com](http://www.alexionpharma.com) and clicking on the clinical trials link.

## **About aHUS**

aHUS is a chronic, ultra-rare disease characterized by thrombotic microangiopathy (TMA), the formation of blood clots in small blood vessels throughout the body, causing a reduction in platelet count (thrombocytopenia) and life-threatening damage to the kidney, brain, heart and other vital organs.<sup>5-7</sup> Approximately 60 percent of patients with aHUS require dialysis, a kidney transplant or die within a year of diagnosis, despite currently available care.<sup>2</sup> The majority of patients with aHUS who receive a kidney transplant experience severe complications of the disease, and more than 90 percent of these patients experience failure of the donor kidney.<sup>8</sup>

aHUS is caused by uncontrolled activation of the complement system. When naturally occurring complement inhibitors are absent or do not function normally, the complement system becomes chronically uncontrolled, causing ongoing inflammation and blood clots in vital organs<sup>1,9</sup> In patients with aHUS, uncontrolled complement activation results in an ongoing risk of sudden and catastrophic life-threatening complications.

## **About Soliris**

Soliris (eculizumab) is not approved for the treatment of patients with aHUS and is being provided to patients in clinical studies on an investigational basis. Soliris has been approved by the healthcare authorities in the United States, European Union, Japan and other countries as the first treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH), a rare, debilitating and life-threatening blood disorder defined by hemolysis, or the destruction of red blood cells. Prior to these approvals, there was no therapy specifically available for the treatment of PNH. Soliris is a first-in-class terminal complement inhibitor developed from the laboratory through regulatory approval and commercialization by Alexion.

Patients with PNH in more than 20 countries now have access to Soliris therapy through national or private healthcare providers. As the first terminal complement inhibitor to be approved in countries around the world for any indication, Soliris represents a long-sought breakthrough in medical innovation. Alexion's innovative approach to complement inhibition has received some of the pharmaceutical industry's highest honors: the 2008 Prix Galien USA Award for Best Biotechnology Product with broad implications for future biomedical research, and the 2009 Prix Galien France Award in the category of Drugs for Rare Diseases. More information on Soliris is available at [www.soliris.net](http://www.soliris.net).

## **Important Safety Information**

Soliris is generally well tolerated in patients with PNH. The most frequent adverse events observed in clinical studies of patients with PNH were headache, nasopharyngitis (runny nose), back pain and nausea. Treatment with Soliris should not alter anticoagulant management because the effect of withdrawal of anticoagulant therapy during Soliris treatment has not been established.

The U.S. product label for Soliris also includes a boxed warning: “Soliris increases the risk of meningococcal infections. Meningococcal infection may become rapidly life-threatening or fatal if not recognized and treated early. Vaccinate patients with a meningococcal vaccine at least two weeks prior to receiving the first dose of Soliris; revaccinate according to current medical guidelines for vaccine use. Monitor patients for early signs of meningococcal infections, evaluate immediately if infection is suspected, and treat with antibiotics if necessary.” During PNH clinical studies, two out of 196 vaccinated PNH patients treated with Soliris experienced a serious meningococcal infection. Prior to beginning Soliris therapy, all patients and their prescribing physicians are encouraged to enroll in the PNH Registry, which is part of a special risk-management program that involves initial and continuing education and long-term monitoring for detection of new safety findings.

#### **About Alexion**

Alexion Pharmaceuticals, Inc. is a biopharmaceutical company working to develop and deliver life-changing drug therapies for patients with serious and life-threatening medical conditions. Alexion is engaged in the discovery, development and commercialization of therapeutic products aimed at treating patients with a wide array of severe disease states, including hematologic and kidney diseases, transplant, other inflammatory disorders, and cancer. Soliris (eculizumab) is Alexion’s first marketed product. Alexion is evaluating other potential indications for eculizumab as well as other formulations of eculizumab for additional clinical indications, and is pursuing development of other antibody product candidates in early stages of development. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at: [www.alexionpharma.com](http://www.alexionpharma.com).

#### **Safe Harbor Statement**

*This news release contains forward-looking statements, including statements related to anticipated clinical development milestones and potential health and medical benefits of Soliris (eculizumab) for the potential treatment of patients with aHUS. Forward-looking statements are subject to factors that may cause Alexion’s results and plans to differ from those expected, including for example, decisions of regulatory authorities regarding marketing approval or material limitations on the marketing of Soliris for its current or potential new indications, the possibility that results of published reports or clinical trials are not predictive of safety and efficacy results of Soliris in broader patient populations, the risk that clinical trials may not be completed successfully, the possibility that initial results of commercialization are not predictive*

of future rates of adoption of Soliris, the risk that third parties won't agree to license any necessary intellectual property to Alexion on reasonable terms or at all, the risk that third party payors will not reimburse for the use of Soliris at acceptable rates or at all, and a variety of other risks set forth from time to time in Alexion's filings with the Securities and Exchange Commission, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended June 30, 2010, and in Alexion's other filings with the Securities and Exchange Commission. Alexion does not intend to update any of these forward-looking statements to reflect events or circumstances after the date hereof, except when a duty arises under law.

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- (3) Abstract 1338 entitled "Safety and Efficacy of Eculizumab in aHUS Patients Resistant to Plasma Therapy: Interim Analysis from a Phase II Trial," presented in an oral presentation at the American Society of Nephrology (ASN) Annual Meeting on Saturday, November 20, 2010 at 5:30 p.m. by Dr. Christophe Legendre.
- (4) Abstract 157 entitled "Safety and Efficacy of Eculizumab in aHUS Patients on Chronic Plasma Therapy: Interim Analysis of a Phase II Trial," presented in a poster presentation at the American Society of Nephrology (ASN) Annual Meeting on Friday, November 19, 2010 from 10:00 a.m.-2:30 p.m. by Dr. Petra Muus.
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