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

## FORM 8-K

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

Date of report (Date of earliest event reported): September 23, 2011

# ALEXION PHARMACEUTICALS, INC.

(Exact name of registrant as specified in its charter)

	Delaware	000-27756	13-3648318
	(State or other jurisdiction of of incorporation or organization)	(Commission File Number)	(I.R.S. Employer Identification No.)
		352 Knotter Drive, Cheshire, Connecticut (	6410
	_	(Address of Principal Executive Offices) (Zip Code)	
		Registrant's telephone number, including area code:	(203) 272-2596
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 ( <i>see</i> General Instruction A.2. below):			
	Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)		
	Soliciting material pursuant to Rul	rial pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)	
	Pre-commencement communication	ions pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))	
	Pre-commencement communication	nent communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))	

#### Item 8.01 Other Events.

(a) Receipt of CHMP Recommendation for Soliris for the Treatment of Patients with aHUS.

On September 23, 2011, Alexion Pharmaceuticals, Inc. issued a press release announcing that the European Committee for Medicinal Products for Human Use (CHMP) has adopted a positive opinion recommending that the therapeutic indication for Soliris® (eculizumab) be extended to include the treatment of patients with atypical hemolytic uremic syndrome (aHUS). A copy of the press release is filed as Exhibit 99.1 to this Current Report on Form 8-K.

(b) Receipt of FDA Marketing Authorization for Soliris for the Treatment of Patients with aHUS.

On September 23, 2011, Alexion issued a press release announcing that the U.S. Food and Drug Administration has approved Soliris for the treatment of all pediatric and adult patients with aHUS. A copy of the press release is filed as Exhibit 99.2 to this Current Report on Form 8-K.

#### Item 9.01 Financial Statements and Exhibits.

- (d) Exhibits
- 99.1 Press Release issued by Alexion Pharmaceuticals, Inc. on September 23, 2011 relating to the recommendation of CHMP.
- 99.2 Press Release issued by Alexion Pharmaceuticals, Inc. on September 23, 2011 relating to receipt of FDA marketing authorization.

### 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.

Date: September 23, 2011

### ALEXION PHARMACEUTICALS, INC.

By: /s/ Michael V. Greco

Name: Michael V. Greco

Title: Associate General Counsel and Corporate Secretary



# Alexion Receives CHMP Positive Opinion for Soliris® (eculizumab) in Patients with Atypical Hemolytic Uremic Syndrome (aHUS) in the European Union

— CHMP recommends marketing authorization for Soliris to treat all patients with a HUS —

CHESHIRE, Conn. — September 23, 2011 — Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) today announced that the European Committee for Medicinal Products for Human Use (CHMP) has adopted a positive opinion recommending that the therapeutic indication for Soliris® (eculizumab) be extended to include the treatment of pediatric and adult patients with atypical hemolytic uremic syndrome (aHUS) in Europe. Based on the CHMP's positive recommendation, a final decision from the European Commission is expected in approximately two months, after which the Company will then begin the country-by-country reimbursement processes.

There are no therapies approved for the treatment of aHUS in Europe. aHUS is an ultra-rare, life-threatening, genetic disease that progressively damages vital organs, leading to stroke, heart attack, kidney failure and death. The morbidity and premature mortality in aHUS is caused by chronic uncontrolled activation of the complement system, resulting in the formation of blood clots in small blood vessels throughout the body, known as thrombotic microangiopathy or TMA. Despite current supportive care, more than half of all patients with aHUS die, require kidney dialysis or have permanent kidney damage within 1 year of diagnosis.

"The positive opinion adopted by the CHMP is a significant step towards making Soliris available to children and adults suffering with aHUS in Europe," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "Importantly, the adopted CHMP opinion recommends to extend marketing authorization for Soliris to treat all patients with aHUS. We look forward to efficiently working with authorities to make Soliris broadly available for European patients suffering with aHUS."

The CHMP based its opinion on clinical data from two prospective pivotal Phase 2 open-label clinical trials in adolescent and adult patients with aHUS, and a third retrospective study in children, adolescents, and adults with aHUS. A summary of the CHMP opinion can be accessed at http://www.emea.europa.eu.

Soliris is approved in the US (2007), European Union (2007), Japan (2010) and in other territories, for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH), a debilitating, ultra-rare and life-threatening blood disorder. Alexion has also filed a supplemental Biologics License Application (sBLA) for Soliris as a treatment for patients with aHUS with the U.S. Food and Drug Administration (FDA).

#### **About aHUS**

aHUS is a chronic, ultra-rare, and life-threatening disease in which a genetic deficiency in one or more complement regulatory genes causes life-long uncontrolled complement activation, resulting in complement-mediated thrombotic microangiopathy (TMA), the formation of blood clots in small blood vessels throughout the body. Permanent, uncontrolled complement activation in aHUS causes a life-long risk for TMA, which leads to sudden, catastrophic, and life-threatening damage to the kidney, brain, heart, and other vital organs, and premature death. And of all patients with aHUS die, require kidney dialysis or have permanent kidney damage within 1 year of diagnosis. Patients with aHUS who receive a kidney transplant commonly experience subsequent systemic TMA, resulting in a 90% transplant failure rate.

aHUS affects both children and adults. In a large group of aHUS patients, 60% were first diagnosed at younger than 18 years of age.<sup>6</sup> Complement-mediated TMA also causes reduction in platelet count (thrombocytopenia) and red blood cell destruction (hemolysis). While mutations have been identified in at least ten different complement regulatory genes, mutations are not identified in 30-50% of patients with a confirmed diagnosis of aHUS.<sup>6</sup>

#### **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, a debilitating, ultra-rare and life-threatening blood disorder defined by chronic uncontrolled complement activation which causes chronic red blood cell destruction (hemolysis), leading to blood clots, organ failure, and shortened survival. Prior to these approvals, there were no therapies specifically available for the treatment of patients with PNH. Soliris (eculizumab) is not approved for

the treatment of aHUS or any indication other than PNH. Alexion's breakthrough 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

#### **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 of patients with PNH with Soliris should not alter anticoagulant management because the effect of withdrawal of anticoagulant therapy during Soliris treatment has not been established.

In Europe, the product label for Soliris includes a special warning: "Due to its mechanism of action, the use of Soliris increases the patient's susceptibility to meningococcal infection (Neisseria meningitidis). These patients might be at risk of disease by uncommon serogroups (particularly Y, W135 and X), although meningococcal disease due to any serogroup may occur. To reduce the risk of infection, all patients must be vaccinated at least 2 weeks prior to receiving Soliris and must be re-vaccinated according to current medical guidelines for vaccination use. Tetravalent vaccines against serotypes A, C, Y and W135 are strongly recommended, preferably conjugated ones. Vaccination may not be sufficient to prevent meningococcal infection. Consideration should be given to official guidance on the appropriate use of antibacterial agents. Cases of serious or fatal meningococcal infections have been reported in Soliris treated patients. All patients should be monitored for early signs of meningococcal infection, evaluated immediately if infection is suspected, and treated with antibiotics if necessary. Patients should be informed of these signs and symptoms and steps taken to seek medical care immediately."

The U.S. product label for Soliris also includes a boxed warning: "Life-threatening and fatal meningococcal infections have occurred in patients treated with Soliris. Meningococcal infection may become rapidly life-threatening or fatal if not recognized and treated early. Comply with the most current Advisory Committee on Immunization Practices (ACIP) recommendations for meningococcal vaccination in patients with complement deficiencies. Immunize patients with a meningococcal vaccine at least 2 weeks prior to administering the first dose of Soliris, unless the risks of delaying Soliris therapy outweigh the risk of developing a meningococcal infection. (See Serious Meningococcal Infections (5.1) for additional guidance on the management of meningococcal infection.) Monitor patients for early signs of meningococcal infections and evaluate immediately if infection is suspected. Soliris is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS). Under the Soliris REMS, prescribers must enroll in

the program (5.2). Enrollment in the Soliris REMS program and additional information are available by telephone: 1-888-soliris (1-888-765-4747)."

Prior to beginning Soliris therapy, all patients and their prescribing physicians in the United States and Europe will be enrolled in the Soliris Safety 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.

In the United States, please see full prescribing information for Soliris at www.soliris.net.

#### **About Alexion**

Alexion Pharmaceuticals, Inc. is a biopharmaceutical company focused on serving patients with severe and ultra-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, a debilitating, ultra-rare and life-threatening blood disorder. Soliris is approved in more than 35 countries. Alexion is evaluating other potential indications for Soliris and is pursuing development of other innovative biotechnology product candidates in early stages of development. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at: www.alexionpharma.com.

#### **Safe Harbor Statement**

This news release contains forward-looking statements, including statements related to anticipated clinical development, regulatory and commercial 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, 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, 2011, 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.

[ALXN-G]

#### References

- 1. Noris M, Remuzzi G: Atypical hemolytic-uremic syndrome. N Engl J Med 2009 361:1676-87
- 2. Benz K, Amann K. Thrombotic microangiopathy: new insights. Curr Opin Nephrol Hypertens 2010 May;19(3):242-7
- 3. Tsai HM. The molecular biology of thrombotic microangiopathy. Kidney Int 2006 Jul;70(1):16-23.
- 4. Caprioli J, Noris M, Brioschi S, et al; for the International Registry of Recurrent and Familial HUS/TTP. Genetics of HUS: the impact of MCP, CFH, and IF mutations on clinical presentation, response to treatment, and outcome. Blood. 2006;108:1267-1279.
- 5. Bresin E, Daina E, Noris M, et al; International Registry of Recurrent and Familial HUS/TTP. Outcome of renal transplantation in patients with non–Shiga toxin-associated hemolytic uremic syndrome: prognostic significance of genetic background. Clin J Am Soc Nephrol. 2006;1:88-99.
- 6. Noris M, Caprioli J, Bresin E, et al. Relative role of genetic complement abnormalities in sporadic and familial aHUS and their impact on clinical phenotype. Clin J Am Soc Nephrol. 2010;5:1844-1859.



# Soliris® (eculizumab) Approved by FDA for All Patients with Atypical Hemolytic Uremic Syndrome (aHUS)

— First and Only Approved Treatment for Children and Adults Suffering with aHUS, an Ultra-Rare, Life-Threatening Disease —

— Conference Call Scheduled for Monday, September 26, 2011 at 10:00am Eastern —

CHESHIRE, Conn. — September 23, 2011 — Alexion Pharmaceuticals, Inc. (Nasdaq: ALXN) today announced that the U.S. Food and Drug Administration (FDA) has approved Soliris® (eculizumab) for the treatment of all pediatric and adult patients with atypical hemolytic uremic syndrome (aHUS). aHUS is an ultra-rare, life-threatening, genetic disease that progressively damages vital organs, leading to stroke, heart attack, kidney failure and death.¹ The morbidity and premature mortality in aHUS is caused by chronic uncontrolled activation of the complement system, resulting in the formation of blood clots in small blood vessels throughout the body, known as thrombotic microangiopathy or TMA.².³ Despite current supportive care, more than half of all patients with aHUS die, require kidney dialysis or have permanent kidney damage within 1 year of diagnosis.⁴

Soliris, a first-in-class terminal complement inhibitor, specifically targets uncontrolled complement activation, and is indicated for the treatment of patients with aHUS to inhibit complement-mediated TMA. The new aHUS indication has been granted under the FDA's accelerated approval process based on two prospective studies in a total of 37 adult and adolescent patients, together with a retrospective study that included 19 pediatric patients. The effectiveness of Soliris in aHUS is based on the effects on TMA and renal function in these completed trials. Prospective clinical trials in additional patients are ongoing to confirm the benefit of Soliris in patients with aHUS. The ongoing prospective clinical trials are designed with the same endpoints as the completed trials. Soliris is not indicated for the treatment of patients with Shiga toxin E coli-related hemolytic uremic syndrome (STEC-HUS).<sup>5</sup> Alexion is currently evaluating the safety and efficacy of eculizumab for the treatment of patients with STEC-HUS.

"Soliris directly targets uncontrolled complement activation, the underlying cause of the progressive organ failure and shortened life span of patients with aHUS, an extremely rare and devastating disease," said Larry Greenbaum, M.D., Ph.D., Director of Pediatric Nephrology at Emory University and Children's Healthcare of Atlanta. "The FDA approval of Soliris in aHUS marks the most important advance that has been made for patients and families with this disease."

"In clinical trials, Soliris markedly decreased the TMA process, which is responsible for thrombosis, renal impairment, seizures, and angina in patients with aHUS," said Craig B. Langman, M.D., The Isaac A Abt MD Professor of Kidney Diseases, Head of Kidney Diseases, Feinberg School of Medicine, Northwestern University. "This is the first time I have seen a therapy with such a dramatic benefit, including restored kidney function. Soliris can change the course of aHUS and make a remarkable difference for patients with this life-threatening disease."

Earlier today, Alexion announced that the European Committee for Medicinal Products for Human Use (CHMP) has adopted a positive opinion recommending that the therapeutic indication for Soliris be extended to include the treatment of pediatric and adult patients with aHUS. Based on the CHMP's positive recommendation, a final decision from the European Commission is expected in approximately two months. Soliris has been previously approved in the US (2007), European Union (2007), Japan (2010) and in other territories, for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH), a debilitating, ultra-rare and life-threatening blood disorder.

"Soliris is the treatment advance that the aHUS community has been seeking for decades," said Bill Biermann, Co-founder of the Foundation for Children with Atypical HUS. "Today's approval is a breakthrough for patients and their families who have been waiting for a treatment for this extremely rare genetic disease. Greater awareness and understanding, along with an effective new therapy, has the potential to accelerate diagnosis and improve management of aHUS, sparing patients and families the devastation many of us have endured."

#### Soliris in aHUS Clinical Data

The FDA approval of Soliris in aHUS is based on data from two prospective pivotal Phase 2 open-label clinical trials in adolescent and adult patients with aHUS, and a third retrospective study in children, adolescents, and adults with aHUS. The studies included patients with or without an identified complement regulatory factor genetic mutation. Patients had an ADAMTS13 activity level greater than 5%. The three studies included: (i) 17 patients who were resistant or intolerant to plasma exchange/infusion, (ii) 20 patients who were receiving chronic plasma exchange/infusion, and (iii) 19 pediatric patients (ages 2 months to 17 years) who received Soliris outside of prospective clinical trials and with or without prior plasma exchange/infusion. All patients treated with Soliris demonstrated reduction in terminal complement activity. All studies met their key clinical

objectives. Final data from the prospective studies were presented at the 16th Congress of the European Hematology Association (EHA) in June 2011.6.7

- In the first study<sup>6</sup>, Soliris-treated patients demonstrated a significant improvement in platelet count from baseline through week 26 of 73×10<sup>9</sup>/L (p=0.0001). Hematologic normalization was observed in 13 of 17 Soliris-treated patients (76%). TMA event-free status was achieved by 15 of 17 Soliris-treated patients (87%). Patients treated with Soliris also showed a statistically significant reduction in the TMA intervention rate, improved renal function, reduction in dialysis, and improved quality of life.
- In the second study<sup>7</sup>, the primary endpoint of TMA event-free status was achieved by 16 of 20 Soliris-treated patients (80%). Hematologic normalization was achieved in 18 of 20 Soliris-treated patients (90%). Patients treated with Soliris also achieved statistically significant reduction in the TMA intervention rate, maintained or improved kidney function, and improved quality of life. No patient required new dialysis with Soliris.
- In the third study, as described in the new Soliris product label, platelet count was normalized in 17 of 19 pediatric patients (89%) treated with Soliris. Patients treated with Soliris also achieved a reduction in the TMA intervention rate. No patient required new dialysis during treatment with Soliris. The safety and effectiveness of Soliris for the treatment of aHUS appeared similar in pediatric and adult patients.

Soliris was well tolerated in these clinical studies. The most frequently reported adverse events were hypertension, upper respiratory tract infection, and diarrhea.

"Today's approval brings life-transforming hope to children and adults suffering with aHUS," said Leonard Bell, M.D., Chief Executive Officer of Alexion. "In a disease defined by uncontrolled complement activation, it is important to note that in our clinical studies, every patient with aHUS had an objective reduction in complement activity with Soliris therapy. We are pleased that this important treatment is now available for patients with aHUS and their families."

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

Alexion will host a conference call/webcast that is scheduled for Monday, September 26, 2011, at 10:00 a.m., Eastern Time to discuss the FDA approval. In addition, a brief financial update will be provided. To participate in this call, dial 877-719-9801 (USA) or 719-325-4806 (International), confirmation code 4412871 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 888-203-1112 (USA) or 719-457-0820 (International), confirmation code 4412871. The audio webcast can be accessed at www.alexionpharma.com.

#### About aHUS

aHUS is a chronic, ultra-rare, and life-threatening disease in which a genetic deficiency in one or more complement regulatory genes causes life-long uncontrolled complement activation, resulting in complement-mediated thrombotic microangiopathy (TMA), the formation of blood clots in small blood vessels throughout the body. Permanent, uncontrolled complement activation in aHUS causes a life-long risk for TMA, which leads to sudden, catastrophic, and life-threatening damage to the kidney, brain, heart, and other vital organs, and premature death. <sup>2,3</sup> More than half of all patients with aHUS die, require kidney dialysis or have permanent kidney damage within 1 year of diagnosis. Patients with aHUS who receive a kidney transplant commonly experience subsequent systemic TMA, resulting in a 90% transplant failure rate.

aHUS affects both children and adults. In a large group of aHUS patients, 60% were first diagnosed at younger than 18 years of age. Complement-mediated TMA also causes reduction in platelet count (thrombocytopenia) and red blood cell destruction (hemolysis). While mutations have been identified in at least ten different complement regulatory genes, mutations are not identified in 30-50% of patients with a confirmed diagnosis of aHUS.

#### **About Soliris**

Soliris is a first-in-class terminal complement inhibitor developed from the laboratory through regulatory approval and commercialization by Alexion. Soliris is approved in the U.S., European Union, Japan and other countries as the first treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH), a debilitating, ultra-rare and life-threatening blood disorder, characterized by complement-mediated hemolysis (destruction of red blood cells). Soliris is also approved in the U.S. as the first treatment for patients with atypical Hemolytic Uremic Syndrome (aHUS), a debilitating, ultra-rare and life-threatening genetic disorder characterized by complement-mediated thrombotic microangiopathy (blood clots in small vessels).

Alexion's breakthrough approach in complement inhibition has received 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.

#### **Important Safety Information**

Soliris is generally well tolerated in patients with PNH and aHUS. In patients with PNH, the most frequently reported adverse events observed with Soliris treatment in clinical studies were headache, nasopharyngitis (runny nose), and back pain. Soliris treatment of patients with PNH should not alter anticoagulant management because the effect of withdrawal of anticoagulant

therapy during Soliris treatment has not been established. In patients with aHUS, the most frequently reported adverse events observed with Soliris treatment in clinical studies were hypertension, upper respiratory tract infection, and diarrhea.

The U.S. product label for Soliris also includes a boxed warning: "Life-threatening and fatal meningococcal infections have occurred in patients treated with Soliris. Meningococcal infection may become rapidly life-threatening or fatal if not recognized and treated early. Comply with the most current Advisory Committee on Immunization Practices (ACIP) recommendations for meningococcal vaccination in patients with complement deficiencies. Immunize patients with a meningococcal vaccine at least 2 weeks prior to administering the first dose of Soliris, unless the risks of delaying Soliris therapy outweigh the risk of developing a meningococcal infection. (See Serious Meningococcal Infections (5.1) for additional guidance on the management of meningococcal infection.) Monitor patients for early signs of meningococcal infections and evaluate immediately if infection is suspected. Soliris is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS). Under the Soliris REMS, prescribers must enroll in the program (5.2). Enrollment in the Soliris REMS program and additional information are available by telephone: 1-888-soliris (1-888-765-4747)."

Please see full prescribing information for Soliris, including boxed WARNING regarding risk of serious meningococcal infection.

#### **About Alexion**

Alexion Pharmaceuticals, Inc. is a biopharmaceutical company focused on serving patients with severe and ultra-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 more than 35 countries for the treatment of PNH, and in the United States for the treatment of aHUS. Alexion is evaluating other potential indications for Soliris and is pursuing development of other innovative biotechnology product candidates in early stages of development. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at: www.alexionpharma.com.

#### **Safe Harbor Statement**

This news release contains forward-looking statements, including statements related to anticipated clinical development, regulatory and commercial 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, 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, 2011, 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.

[ALXN-G]

#### References

- 1. Noris M, Remuzzi G: Atypical hemolytic-uremic syndrome. N Engl J Med 2009 361:1676-87
- 2. Benz K, Amann K. Thrombotic microangiopathy: new insights. Curr Opin Nephrol Hypertens 2010 May;19(3):242-7
- 3. Tsai HM. The molecular biology of thrombotic microangiopathy. Kidney Int 2006 Jul;70(1):16-23.
- 4. Caprioli J, Noris M, Brioschi S, et al; for the International Registry of Recurrent and Familial HUS/TTP. Genetics of HUS: the impact of MCP, CFH, and IF mutations on clinical presentation, response to treatment, and outcome. Blood. 2006;108:1267-1279.
- 5. Soliris (eculizumab) full prescribing information. 2011. Cheshire, CT: Alexion Pharmaceuticals
- 6. Loirat C, Babu S, Furman R, Sheerin N, Cohen D, Gaber O, et al. Eculizumab Efficacy and Safety in Patients With Atypical Hemolytic Uremic Syndrome (aHUS) Resistant to Plasma Exchange/Infusion [poster]. Presented at the 16th Congress of European Hematology Association (EHA), 2011. London, UK.
- 7. Loirat C, Muus P, Legendre C, Douglas K, Hourmant M, Delmas Y, et al. A Phase II Study of Eculizumab in Patients With Atypical Hemolytic Uremic Syndrome Receiving Chronic Plasma Exchange/Infusion [poster]. Presented at the 16th Congress of European Hematology Association (EHA), 2011. London, UK.
- 8. Bresin E, Daina E, Noris M, et al; International Registry of Recurrent and Familial HUS/TTP. Outcome of renal transplantation in patients with non–Shiga toxin-associated hemolytic uremic syndrome: prognostic significance of genetic background. Clin J Am Soc Nephrol. 2006;1:88-99.
- 9. Noris M, Caprioli J, Bresin E, et al. Relative role of genetic complement abnormalities in sporadic and familial aHUS and their impact on clinical phenotype. Clin J Am Soc Nephrol. 2010;5:1844-1859.