



## **Regulated Information**

**Disclosure in accordance with the law of May 2, 2007**

### **ThromboGenics NV – Business Update**

**Leuven, Belgium – 3 November 2011** – ThromboGenics NV (Euronext Brussels: THR), a biopharmaceutical company focused on developing innovative ophthalmic medicines, today issued a business update and its nine-month financial results ending 30 September, 2011.

#### **Highlights (including post-period events)**

##### **Ocriplasmin**

- European Medicines Agency (EMA) accepts for review the ocriplasmin Marketing Authorisation Application (MAA) for treatment of symptomatic vitreomacular adhesion (VMA) including macular hole
- Preparation of U.S. ocriplasmin filing with the FDA is on target and submission is planned before the end of 2011
- Leading retina specialists present ocriplasmin Phase III data, including responder analysis, at the American Academy of Ophthalmology (AAO), the world's largest ophthalmology conference. Ocriplasmin featured as the opening talk in the first scientific session of the Retina Subspecialty Day and also in Best of the Posterior Segment Specialty Meetings

##### **Antibody candidates**

- Recruitment progressing well in a 600-patient Phase IIb trial with TB-402 (anti-Factor VIII antibody) for the prevention of venous thromboembolism (VTE) after total hip replacement surgery
- Our partner Roche has started patient recruitment into two Phase Ib/II trials with the novel human anticancer antibody TB-403 (anti-PIGF)

##### **Financial**

- €88.3 million in cash and cash investments as of 30 September 2011, compared with €59.1 million at the end of September 2010
- Reported revenues of €2.5 million in the first nine months of 2011 versus €6.1 million in the first nine months of 2010

##### **Dr Patrik De Haes, CEO of ThromboGenics, commenting on today's update said:**

"We are delighted that the EMA has accepted the European Marketing Authorisation Application for ocriplasmin for review. We are now focused on filing the Biologics License Application (BLA) with the FDA before the end of the year. If approved, ocriplasmin would be the first pharmacological treatment for symptomatic VMA including macular hole. This would be a major advance in treating a condition that can only currently be treated with surgery, usually after the patient's sight has deteriorated significantly.

"We are extremely encouraged by the growing interest in ocriplasmin among the retina community in the USA and Europe, as physicians recognize the advantages of having a pharmacological option to treat this progressive retinal condition earlier. The growing advocacy, combined with the growing momentum of our commercial activities, gives me



confidence that our ocriplasmin-based ophthalmic strategy will deliver value for the company and its shareholders, as well as important clinical benefits for retina specialists and patients.”

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**Business highlights**

**Ocriplasmin – Crucial to building a successful ophthalmology business**

- **EMA accepts ocriplasmin Marketing Authorisation Application (MAA) for review**
- **ThromboGenics is on track to file the ocriplasmin biological license application (BLA) with the FDA before the end of 2011, as previously stated**
- **Leading retinal experts present ocriplasmin Phase III data, including responder analysis, at American Academy of Ophthalmology (AAO)**
- **ICD-9 disease code for VMA in the U.S. effective as of October 2011**
- **Appointment of Dr David Shima as Chief Scientific Officer, Ophthalmology to drive ophthalmology pipeline development**

**Accepted Filing with the EMA and planned BLA submission in the U.S.**

ThromboGenics has successfully filed the MMA for ocriplasmin (2.5 mg/ml, solution for injection) with the European Medicines Agency (EMA) for the treatment of symptomatic vitreomacular adhesion (VMA) including macular hole, and it has been accepted for review.

The MAA is being evaluated through the EMA's Centralised Procedure with an official start date of October 19, 2011. If approved, ocriplasmin will gain marketing authorization for all EU Member States simultaneously.



In addition, ThromboGenics is preparing to file a BLA with the FDA before the end of 2011 as planned.

These filings could enable the first launch of ocriplasmin before the end of 2012, based on a successful regulatory approval.

Symptomatic VMA is a progressive condition that if left untreated generally leads to retinal distortion, further decrease in vision and has potential for irreversible damage and complications. If approved, ocriplasmin will be the first pharmacological treatment for a condition which is currently only treated when the disease progresses to significant complications and symptoms that warrant surgery.

ThromboGenics' own extensive market research confirms the high unmet need in the treatment of symptomatic VMA and the significant commercial potential of ocriplasmin, with approximately 500,000 patients in the U.S. and the major EU markets estimated to be eligible for treatment at the time of launch.

ThromboGenics is investing heavily into building a strong commercial organization to successfully launch ocriplasmin on its own and target approximately 4,000 retinal specialists that treat these patients across the U.S. and the five largest markets in the EU. The Company is continuing to grow its commercial and medical affairs teams by recruiting individuals with significant expertise in new product launch and strong relationships with the target retina physicians.

### **Ocriplasmin Phase III data presented at key AAO slots**

ThromboGenics has continued to raise awareness about ocriplasmin and its Phase III data for the treatment of symptomatic VMA including macular hole. In October, three leading retina experts presented ocriplasmin Phase III data, including responder analysis, to an international audience at the AAO, the world's largest gathering of retinal physicians:

Dr Julia Haller presented the ocriplasmin Phase III data including the responder analysis at the opening session of the AAO Retina Subspecialty Day.

Dr Peter Kaiser was invited to present his paper on ocriplasmin for the treatment of macular holes at the Best of the Posterior Segment Specialty Meetings. This presentation was selected by the AAO Special Projects Committee based on the best data presentations from major retina meetings during 2011. Dr Kaiser previously presented a review of these data at the American Society of Retina Specialists.

Dr Pravin Dugel also highlighted the benefits of a pharmacological treatment at the AAO General Session.

Presentations of ocriplasmin data were also made this quarter at the German Society of Ophthalmology conference in Berlin and the Retina Society meeting in Rome.

ThromboGenics intends to present further presentations at key international conferences until launch to continue building ocriplasmin's profile among the global retina specialist community. The next major upcoming conferences include:

- **British Eire Vitreoretinal Society Meeting** (Canterbury, UK 9-11 November):
  - Dr Tim Jackson (Kings College, London): Single Intravitreal Ocriplasmin Injection for the Treatment of Vitreomacular Traction: Responder Analysis of the Pivotal Trials



- Dr Heinrich Heinmann (Liverpool, UK): Single Intravitreal Ocriplasmin Injection for the Treatment of Macular Hole: Responder Analysis of the Pivotal Trials
- **Academia Ophthalmologica Belgica** (Brussels, 23-25 November)
  - Dr. Peter Stalmans: Enzymatic Vitreolysis with Ocriplasmin
- **Asia Pacific Vitreo Retinal Society and Vitreo Retinal Society of India** (Hyderabad, 1-3 December)
  - Dr. Pravin Dugel: Single Intravitreal Ocriplasmin Injection for the Treatment of Symptomatic Vitreomacular Adhesion Including Macular Hole
- **International Symposium on Ocular Pharmacology and Therapeutics** (Vienna, 1-4 December)
  - Dr. Peter Kaiser (invited speaker): Treatment of Symptomatic Vitreomacular Adhesions (VMA) With Ocriplasmin: A Subgroup Analysis of the Phase III MIVI-TRUST Program
  - ThromboGenics Lunch Symposium on the Role of Vitreous in Retinal Diseases: Diagnosis and Treatment with Five Leading International Speakers (two from U.S., two from Germany, and one from UK)

#### **New ICD-9-CM disease code effective for the coding of VMA/VMT**

A new disease code recognizing VMA as a separate identifiable vision-threatening condition has been granted in the U.S. at the request of the AAO and ASRS (American Society of Retina Specialists). The new code has been in effect since October 2011.

ICD-9-CM is an official committee that assigns codes to diagnoses and procedures used in the U.S. These codes are used for tracking the prevalence of diseases and reimbursing physician services as it relates to disease management.

#### **Dr David Shima appointed to bring new impetus to ThromboGenics' preclinical ophthalmology research**

ThromboGenics has strengthened its preclinical ophthalmology research efforts with the appointment of a new chief scientific officer. In October 2011, Dr David T. Shima joined as CSO, Ophthalmology to help build the Company's pipeline of new ophthalmic medicines, which are expected to be generated both internally and from in-licensing deals. He reports to CEO Dr Patrik De Haes.

Dr Shima is Professor at UCL's Institute of Ophthalmology (UK) and has held several roles in the ophthalmic industry including CSO of Jerini Ophthalmic Inc. and Senior Vice President of Research and Preclinical Development at Eyetech. He holds a PhD in Cell and Developmental Biology from Harvard.

#### **Antibody candidate clinical trials progress**

#### **TB-402 (anti-Factor VIII antibody) – Patients dosed in Phase IIb Trial with Novel, Long-Acting Anticoagulant**

ThromboGenics, along with its partner BioInvent, is continuing to invest in the clinical development of TB-402, its novel long-acting anticoagulant.



The latest trial is a Phase IIb multicenter, double blind and randomized controlled study evaluating the safety and efficacy of two dose levels of TB-402, given as a single intravenous infusion after total hip surgery, in comparison with the recently approved Factor Xa inhibitor rivaroxaban (Bayer). The trial, which started in April, will enroll 600 patients from approximately 40 centers in Europe. Recruitment of the study is going well, with results expected in the second half of 2012.

### **TB-403 (anti PIGF) – Roche enrolling patients in glioblastoma multiforme and primary liver cancer trials**

Our partner Roche began enrolling patients in a Phase Ib/II trial initiated in May 2011 with our novel anticancer antibody TB-403 in patients with glioblastoma multiforme, the most aggressive form of primary brain cancer.

The start of the trial triggered a €4 million milestone payment to ThromboGenics and partner Biolinvent. The 100-patient multicenter trial will examine the safety and clinical effect of TB-403 in combination with Avastin® (bevacizumab) in patients with recurrent glioblastoma.

Patient recruitment is also under way for a Phase Ib study that Roche initiated in March 2011 with TB-403 in patients with primary liver cancer (hepatocellular carcinoma). This study will determine the safety, tolerability and dosage of TB-403 in combination with Nexavar® (sorafenib), as well as pharmacokinetics and pharmacodynamics. The study will recruit 60-70 patients.

### **Dr Koen Kas appointed new Chief Scientific Officer Oncology**

ThromboGenics appointed Dr Koen Kas as CSO, Oncology in the third quarter of 2011 to advance its preclinical anti-cancer research. He also reports to CEO Dr Patrik De Haes.

Dr Kas has extensive experience in oncology and drug development. He was most recently Founding CSO of Pronota and has held senior roles including Director of Drug Discovery at Galapagos and Director of Oncology at Tibotec-Virco. Dr Kas also has a broad academic career, as Professor Molecular Oncology at the University of Leuven, Visiting Lecturer at Harvard Medical School and Visiting Professor at the University of Ghent. Dr Kas is also Chairman of the Scientific Committee of the European Cancer Prevention Organisation. He has authored more than 50 publications and 20 patent applications. He holds a PhD cum laude in Biomedical Sciences.

### **Financial Update**

As of 30 September 2011, ThromboGenics had €88.3 million in cash and cash investments, compared with €59.1 million on 30 September 2010 and €96.4 million as of 30 June 2011. This level of cash resources is expected to allow ThromboGenics to execute its operational plans until post the launch of ocriplasmin.

ThromboGenics reported €2.5 million in revenues for the nine months to 30 September 2011, versus €6.1 million in the same period in 2010. Nearly all of the revenue in 2011 came from the Company's share of the €4 million milestone payment from Roche after it initiated a Phase Ib/II trial with TB-403 in glioblastoma multiforme. The Company's revenues in 2010 were also largely due to milestone payments from Roche.



R&D expenses were €15.7 million in the first nine months of 2011, versus €12.8 million in the same period in 2010, as the Company continued to invest in the development of its product pipeline, including the Phase IIb trial with TB-402. Half of the costs of this TB-402 study are being met by our co-development partner BioInvent. In addition, €3.8 million of costs related to the ocriplasmin Phase III clinical program have been capitalized. This compares to €7.0 million in the corresponding period in 2010.

- Ends -

### **About ThromboGenics**

ThromboGenics is a biopharmaceutical company focused on developing innovative ophthalmic medicines. The Company's lead product ocriplasmin has successfully completed two Phase III clinical trials for the pharmacological treatment of symptomatic vitreomacular adhesion (VMA). The Marketing Authorisation Application for ocriplasmin has been accepted for review in Europe and the BLA is expected to be filed in the U.S. by the end of 2011. Ocriplasmin is also in Phase II clinical development for additional vitreoretinal conditions.

ThromboGenics is also developing novel antibody therapeutics in collaboration with BioInvent International. These include TB-402 (anti-Factor VIII), a long-acting anticoagulant in Phase II, and TB-403 (anti-PIGF) in Phase Ib/II for cancer in partnership with Roche.

ThromboGenics is headquartered in Leuven, Belgium. The Company is listed on NYSE Euronext Brussels under the symbol THR. More information is available at [www.thrombogenics.com](http://www.thrombogenics.com).

### ***Important information about forward-looking statements***

*Certain statements in this press release may be considered "forward-looking". Such forward-looking statements are based on current expectations, and, accordingly, entail and are influenced by various risks and uncertainties. The Company therefore cannot provide any assurance that such forward-looking statements will materialize and does not assume an obligation to update or revise any forward-looking statement, whether as a result of new information, future events or any other reason. Additional information concerning risks and uncertainties affecting the business and other factors that could cause actual results to differ materially from any forward-looking statement is contained in the Company's Annual Report.*