ThromboGenics Business Update - Q3 2017

Press release
20 October, 2017
Regulated Information

Diabetic Eye Disease programs progressing

€53.7 million cash received from Alcon/Novartis in conjunction with regaining the non-US rights to JETREA

Company's end Q3 2017 cash position €113.4 million

Novartis to invest €10 million in ThromboGenics' share capital

Highlights

- In September, ThromboGenics announced that it regained the non-US rights to JETREA® from Alcon, a Novartis company, based on a mutual agreement that ThromboGenics is a better fit for building a smaller but sustainable long-term business with this unique drug for the treatment of vitreomacular adhesion/ vitreomacular traction. ThromboGenics now owns all rights to JETREA® globally.

- Following the agreement with Alcon/Novartis, **ThromboGenics has €113.4 million in cash**, which will be used to progress its pipeline of **novel disease modifying medicines for the treatment of diabetic eye disease**

- Novartis AG plans to invest **€10 million in ThromboGenics' share capital**

- ThromboGenics is conducting a **Phase I/IIa clinical study evaluating THR-317** (anti-Placental Growth Factor) for Diabetic Macular Edema (DME). The trial was initiated in January 2017 and assesses THR-317's safety and activity in subjects with DME. Study results are expected during Q1 2018.

- ThromboGenics is **conducting a Phase IIa clinical study (CIRCLE) evaluating THR-409 (ocriplasmin)** ability to induce a total posterior vitreous detachment (PVD) in patients with non-proliferative diabetic retinopathy (NPDR) and as a result to prevent progression to proliferative diabetic retinopathy (PDR), a serious sight threatening condition.

- ThromboGenics is making **good progress with its late-stage preclinical pipeline:**
    - **THR-687 (integrin antagonist)** is being developed to treat a broad range of patients with diabetic retinopathy, with or without DME. THR-687 is expected to enter the clinic in H1 2018

    - **THR-149 (selective plasma kallikrein inhibitor)** is being developed to treat edema associated with diabetic retinopathy (DME). THR-149 is also expected to enter the clinic in H1 2018

- In August 2017, **Oncurious** announced an agreement with **VIB** for **expansion of its pipeline** through the acquisition of 5 unique **next generation immuno-oncology (IO)** assets for the treatment of a broad spectrum of cancers

- **Cash and investments were €113.4 million as of the end of September 2017**, compared with €65.1 million at the end of June 2017

Leuven, Belgium - 20 October 2017 - ThromboGenics NV (Euronext Brussels: THR), a biotechnology company developing novel medicines for back of the eye diseases and focused on diabetic eye disease, today issues a business update for the three-month period ending 30 September 2017.

ThromboGenics is developing a competitive and strategic pipeline of disease modifying drug candidates for diabetic eye disease, particularly diabetic retinopathy (DR) and diabetic macular edema (DME).

The current pipeline consists of products with different modes of action, and allows the Company to address the four key segments of the rapidly growing diabetic eye disease market:

- Non-proliferative DR
- Proliferative DR
- Non-proliferative DR with DME
- Proliferative DR with DME

Patrik De Haes, MD ThromboGenics CEO, said*: "The €53 million cash payment that we received as part of our recent agreement with Alcon/Novartis to regain the non-US rights to JETREA® provides us with significant financial resources to pursue our goal of becoming a leader in the treatment of diabetic eye disease. These increased cash resources will allow us to deliver multiple value generating milestones from our pipeline. We are confident that the significant potential of our novel disease modifying drug candidates will continue to be demonstrated over the coming years."*

Progressing Pipeline of Novel Medicines Targeting Diabetic Eye Disease such as Diabetic Retinopathy and Diabetic Macular Edema

Diabetic retinopathy (DR) is the leading cause of visual disability and blindness among professionally active adults. More than one in three people living with diabetes (35.4%) will develop some form of DR in their lifetime. One in five patients with DR presents with sight threatening diabetic macular edema (DME).

ThromboGenics' pipeline comprises of:

- THR-317 - a PLGF inhibitor under development for DME, and potentially as a combination therapy with current anti-VEGF treatments for DME or DR. A Phase I/IIa clinical trial for THR-317 was initiated in January 2017. First results are expected in Q1 2018.
- THR-409 (ocriplasmin) - is being evaluated in a Phase IIa (CIRCLE) clinical study assessing the efficacy and safety of different dose levels of THR-409 to induce a total posterior vitreous detachment (PVD) in patients with non-proliferative diabetic retinopathy (NPDR) and as a result to prevent progression to proliferative diabetic retinopathy (PDR), a serious sight threatening condition.
- THR-687 - an integrin antagonist under development to treat a broad range of patients with DR, with or without DME. THR-687 was in-licensed from Galapagos NV in 2016 and is expected to enter the clinic in H1 2018.
- THR-149 - a selective plasma kallikrein inhibitor under development to treat the edema associated with DR. This compound results from the Company's research collaboration with Bicycle Therapeutics. ThromboGenics has started pivotal toxicology studies and is preparing for clinical development, which is expected to start in H1 2018.

THR-317 - anti-PLGF antibody to treat Diabetic Macular Edema

THR-317 is a potentially disease-modifying PLGF antibody that is being developed to treat DME or DR. For patients who do not respond, or do not respond well to anti-VEGF therapy, THR-317 could potentially be used in combination with anti-VEGF medicines.

ThromboGenics enrolled the first patients in a Phase I/IIa, single-masked, multicenter exploratory study evaluating the safety and efficacy of THR-317 for the treatment of diabetic macular edema (DME) in January 2017.

The Phase I/IIa study is evaluating the safety of three intravitreal injections of two dose levels of THR-317 (4 mg or 8 mg). The trial is also assessing THR-317's activity by evaluating best-corrected visual acuity (BCVA) and central retinal thickness in subjects with DME. The results from the study are expected in Q1 2018.

Earlier this month ThromboGenics' announced the publication of pre-clinical data generated using the murine form of THR-317 in the peer-reviewed ***Experimental Eye Research*** **Journa**l. In its conclusion, the authors confirm THR-317's comparable efficacy to VEGF inhibitors in terms of reducing vascular leakage, and highlighted its potential added ability to reduce inflammation and fibrosis in the mouse eye, without triggering a neurodegenerative response.

In July 2017, ThromboGenics and BioInvent agreed to amend their long-standing agreement, which covers co-development of novel anti-PlGF monoclonal antibody products including THR-317, which ThromboGenics is developing for the treatment of DME. Under the amended arrangement, ThromboGenics gains full and exclusive ownership of THR-317 for development and commercialization in all non-oncology indications. ThromboGenics will continue to carry all costs for development of THR-317 in non-oncology indications, and BioInvent is entitled to five percent royalty on net sales and other revenues.

THR-409 for Non-Proliferative Diabetic Retinopathy - CIRCLE Study

The on-going CIRCLE study is evaluating the ability of multiple doses of THR-409 (ocriplasmin) to induce a total PVD in patients with NPDR. The study is also assessing the safety of multiple doses of THR-409. Recruitment continues to progress steadily but slower than anticipated.

ThromboGenics believes that THR-409 could reduce the risk of disease progression to PDR by inducing a total PVD. Research has suggested that total PVD, a complete separation of vitreous and retina, could prevent the progression of NPDR to PDR.

The CIRCLE study is a Phase II, randomized, double-masked, sham-controlled, multi-center study that evaluates the efficacy and safety of up to 3 intravitreal injections of either 0.125mg or 0.0625mg of THR-409 in subjects with moderate to severe NPDR, to induce total PVD in order to reduce the risk of the patient developing sight-threatening PDR.

The primary endpoint of the CIRCLE study is the percentage of patients with total PVD by the month 3 visit, confirmed by both B-scan ultrasound and SD-OCT.

THR-687 for DR - Further supportive pre-clinical data presented

ThromboGenics is developing THR-687, an integrin antagonist, for the treatment of a broad range of patients with DR, with or without DME.

At the European Association for Vision and Eye Research (EVER) 20th Annual Meeting held in Nice, ThromboGenics delivered a poster presentation entitled "THR-687, a potent small molecule integrin receptor antagonist, holds promise as a therapeutic approach for back-of-the-eye vascular pathologies." The poster provided new pre-clinical evidence supporting the use of THR-687 for the treatment of back-of-the-eye vascular diseases.

The studies presented concluded that THR-687 is a potent and safe treatment, highlighting its ability to inhibit various significant stages in pathologic angiogenesis, an important factor leading to vision loss in DR.

The data presented at EVER provide further support to the development of THR-687 in the treatment of DR ahead of its expected entry into the clinic in the first half of 2018.

JETREA global commercial

ThromboGenics gained the global rights to JETREA® in September 2017. This resulted from an agreement with Alcon, a Novartis company, which included ThromboGenics receiving €53.7 million in cash. Alcon / Novartis will work closely with ThromboGenics to ensure continuity and access to JETREA® for existing and future customers during a transition period of up to two years.

ThromboGenics pioneered the pharmacological vitreolysis drug class through the development of JETREA®, the only pharmacological vitreolysis drug approved for the treatment of symptomatic vitreomacular adhesion (in the US) and vitreomacular traction (in Europe and elsewhere) in over 54 countries globally. Since its first introduction in early 2013, nearly 30,000 patients have been treated with JETREA®.

Oncurious update

Clinical Update: TB-403 for Pediatric Brain Cancers

A Phase I/IIa study was initiated with TB-403 in May 2016, for which patient recruitment is ongoing. The study, conducted by Beat Childhood Cancer (formerly known as the Neuroblastoma and Medulloblastoma Translational Research Center - NMTRC), aims to recruit 27 patients with Relapsed or Refractory Medulloblastoma. The first 2 out of a planned 4 patient groups have now been recruited.

TB-403 is a humanized monoclonal antibody against placental growth factor (PLGF). PLGF is expressed in several types of cancer, including medulloblastoma. High expression of the PLGF receptor neuropilin-1 has been shown to correlate with poor overall survival.

In January 2017, the European Commission confirmed orphan drug designation for TB-403 for medulloblastoma. The orphan designation allows a pharmaceutical company to benefit from incentives from the European Union to develop a medicine for a rare disease, such as reduced fees and protection from competition once the medicine is on the market.

Medulloblastoma is the most common pediatric malignant brain tumor, accounting for 20% of all brain tumors in children. Treatment with TB-403 in relevant animal models for medulloblastoma has demonstrated beneficial effects on tumor growth and survival.

TB-403 is being developed by Oncurious in conjunction with BioInvent International.

In July, ThromboGenics and BioInvent amended their long-standing monoclonal antibody development agreement. In the amended agreement, BioInvent assumes project lead for development of TB-403 in all oncology indications and will increase its share in the economic value of TB-403 from 40% to 50%. Both parties will continue to share equally the costs of developing TB-403 for oncology indications.

Acquisition of next generation immune-oncology assets from VIB

In September 2017, Oncurious announced it had reached an agreement to broaden its portfolio through the acquisition of five immune-oncology assets from VIB. The acquired assets are based on seminal work originating from the VIB-KULeuven labs of Professor Massimiliano Mazzone and Professor Gabriele Bergers, and from the VIB-VUB lab of Professor Jo Van Ginderachter. VIB Discovery Sciences will take the lead in pre-clinical development of these new projects.

With these assets, Oncurious has broadened its pipeline to include preclinical research and drug development programs targeting a broad spectrum of cancers, resulting in an exciting pipeline of next-generation immuno-oncology drugs.

As part of the agreement, VIB will increase its stake in Oncurious, with ThromboGenics remaining the majority shareholder. VIB will also receive a royalty on future sales of any of these assets. ThromboGenics will invest an additional €2.1 million in Oncurious over the next two years.

Financial Update

Cash and investments were €113.4 million as of the end of September 2017, compared with €65.1 million at the end of June 2017.

Additionally, Novartis will invest €10 million in ThromboGenics capital.

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For further information please contact:

ThromboGenics	**Citigate Dewe Rogerson**
Wouter Piepers, Global Head of Corp Coms & Investor Relations +32 16 75 13 10 / +32 478 33 56 32 wouter.piepers@thrombogenics.com	David Dible/Sylvie Berrebi/Isabelle Andrews Tel: +44 20 7282 2867 david.dible@citigatedr.co.uk Sylvie.berrebi@citigatedr.co.uk

About ThromboGenics

ThromboGenics is a biopharmaceutical company focused on developing innovative treatments for eye disease, with a focus on diabetic eye disease. The company's pipeline of disease modifying drug candidates is targeting the key segments of the diabetic eye disease market.

ThromboGenics' clinical pipeline consists of THR-317 and THR-409 (ocriplasmin), both of which were developed from in-house research. THR-317, a PLGF inhibitor, is in a Phase I/IIa clinical study for the treatment of diabetic macular edema, as a stand-alone or as a combination therapy with anti-VEGF treatments. A Phase IIa clinical trial is being conducted to evaluate the safety and efficacy of multiple doses of THR-409 to induce a total Posterior Vitreous Detachment in patients with Non-Proliferative Diabetic Retinopathy (NPDR).

ThromboGenics' pre-clinical pipeline consists of THR-149, a plasma kallikrein inhibitor, which has resulted from research collaboration with Bicycle Therapeutics, and THR-687, an integrin antagonist, which was in-licensed from Galapagos. Both are expected to enter the clinic in H1 2018.

ThromboGenics owns the global rights to JETREA® (ocriplasmin), the only pharmacological vitreolysis drug approved for the treatment of symptomatic vitreomacular adhesion (in the US) and vitreomacular traction (in Europe and elsewhere) in over 54 countries worldwide.

ThromboGenics is headquartered in Leuven, Belgium, and is listed on the NYSE Euronext Brussels exchange under the symbol THR. More information is available at 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.

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