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ThromboGenics Business Update – Q3 2016

Diabetic Eye Disease clinical and pre-clinical programs on track

New Ophthalmic Research presented at EURETINA, EVER and AAO

Company Q3 2016 cash position at 88.2m Euro

Highlights

- ThromboGenics continues to advance its attractive portfolio of **novel medicines for the treatment of diabetic eye disease: 4** novel treatments for **diabetic retinopathy (DR)** - non-proliferative diabetic retinopathy (NPDR) and proliferative diabetic retinopathy (PDR), in the presence or absence of **diabetic macular edema (DME)**

- ThromboGenics is conducting **a Phase IIa clinical study (CIRCLE) evaluating THR-409 (ocriplasmin)** to induce a complete posterior vitreous detachment (PVD) in patients with non-proliferative diabetic retinopathy (NPDR).

- ThromboGenics is developing **THR-317,** an anti-PlGF inhibitor, **for the treatment of DME.** Program is **on track** to enter the clinic by end of 2016.

- Preclinical work with **THR-149**, a plasma kallikrein inhibitor being developed to treat edema associated with diabetic retinopathy (DME), **progressing as planned**

- ThromboGenics signed a **global and exclusive license agreement with Galapagos to develop and commercialize integrin antagonists** for the treatment of diabetic eye disease. The Company has started to explore the potential of these compounds, including **THR-687**, for the treatment of NPDR and PDR

- ThromboGenics presented new preclinical and clinical data at several international ophthalmology expert meetings:
  - The European Society of Retina Specialists (EURETINA)
  - The European Association for Vision and Eye Research (EVER)
  - The American Academy of Ophthalmology (AAO)

- Cash and investments were € 88.2 million as of the end of September 2016, compared with €91.5 million at end of June 2016 and €101.4 million at the end of December 2015

Leuven, Belgium – 20 October 2016 - ThromboGenics NV (Euronext Brussels: THR), a biotechnology company developing novel medicines for diabetic eye disease, today issues a business and financial update for the nine months period ending 30 September 2016.

ThromboGenics is **developing novel medicines for diabetic eye disease**, particularly diabetic retinopathy (DR) and diabetic macular edema (DME).

Diabetes is a major global healthcare problem with 9% of adults (18 years and older) suffering from this disease[1].

Diabetic retinopathy (DR) is the leading cause of visual disability and blindness among professionally active adults[2]. More than one in three living with diabetes (35.4%) will develop some form of DR in their lifetime. One in five patients with DR presents with DME.
DR progresses from mild, non-proliferative to more severe or even proliferative stages. As DR progresses, there is a gradual closure of retinal blood vessels leading to impaired perfusion and ischemia of the retina. When this progresses beyond certain thresholds, severe non-proliferative diabetic retinopathy (NPDR) is diagnosed.

The more advanced stage of DR, Proliferative DR (PDR), is characterized by the development of new blood vessels at the inner surface of the retina as a result of retinal ischemia. These new vessels are prone to bleed, resulting in vitreous hemorrhage. These new vessels may also undergo fibrosis and contraction, which may lead to epiretinal membrane formation, vitreoretinal traction bands, retinal tears and traction or retinal detachments.

PDR is considered high risk when the new vessels are accompanied by vitreous hemorrhage, or when they cover a significant area of the optic disc. Patients who progress to PDR are at high risk of severe vision loss.

ThromboGenics' strategy is focused on developing a strong pipeline of disease modifying drug candidates for diabetic eye diseases:

THR-409 - an ongoing Phase IIa (CIRCLE) clinical study is evaluating the efficacy and safety of multiple doses of ocriplasmin in inducing total posterior vitreous detachment (PVD) in patients with non-proliferative diabetic retinopathy (NPDR).

THR-317 - a PlGF neutralizing monoclonal antibody is being developed for DME and/or for use in combination therapy with current anti-VEGF treatments. THR-317 is expected to enter clinical development by end 2016.

THR-149 - a plasma kallikrein inhibitor is being developed to treat edema associated with diabetic retinopathy. (This compound has resulted from the Company's research collaboration with Bicycle Therapeutics)

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[1] World Health Organization (WHO). (2015). Diabetes. Fact sheet N°312. http://www.who.int/mediacentre/factsheets/fs312/en/ 21 May 2015.

[2] (Cunha-Vaz, 1998; Fong et al., 1999)

THR-687 - a small molecule integrin antagonist being developed to treat a broad range of patients with diabetic retinopathy, with or without DME (in-licensed from Galapagos NV in 2016).

Dr. Patrik De Haes, ThromboGenics' CEO, said*: "We are committed to achieving our goal of developing meaningful new treatments for patients suffering from diabetic eye diseases. With our current cash resources, we can support our activities for the foreseeable future, allowing us to demonstrate the value of our pipeline."*

Research & Development Activities

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

THR-409 for Non Proliferative Diabetic Retinopathy

In January 2016, the Company initiated its Phase IIa (CIRCLE) study.

The CIRCLE study is evaluating the efficacy and safety of multiple doses of THR-409 (ocriplasmin) in inducing total posterior vitreous detachment (PVD) in patients with non-proliferative diabetic retinopathy (NPDR).

Research has suggested that total PVD, a complete separation of vitreous and retina, prevents the progression of NPDR to PDR. This could be explained by total PVD leading to elimination of the scaffold needed for the development of new blood vessels and/or the improvement of oxygen supply to the retina, thereby reducing retinal ischemia, production of VEGF, vascular outgrowth and neovascularization.

The CIRCLE study is a Phase II, randomized, double-masked, sham-controlled, multi-center study that will evaluate the efficacy and safety of up to 3 intravitreal injections of either 0.125mg or half of the approved dose (0.0625mg) of THR-409 in subjects with moderately severe to very severe NPDR, to induce total PVD and reduce the risk of the patient progressing to sight-threatening PDR.

The endpoint of the CIRCLE study is the percentage of patients with total PVD at the time of their visit one month after the 3rd injection, as confirmed by both B-scan ultrasound and SD-OCT.

THR-317 – anti PlGF antibody to treat Diabetic Macular Edema

THR-317 is a potential disease modifying, anti PlGF antibody that has the potential to treat a broad range of patients with late stage diabetic eye disease either alone or in combination with anti-VEGF treatments.

ThromboGenics is currently developing THR-317 for the treatment of diabetic macular edema and expects to start the clinical development of this novel antibody towards the end of 2016.

Oncurious NV – Developing TB-403 for Pediatric Brain Cancers

Oncurious, a subsidiary controlled by ThromboGenics, is developing TB-403 for the treatment of pediatric tumors.

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. 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. The favorable safety profile of TB-403 has already been demonstrated in clinical trials in patients with other diseases.

In March 2016, Oncurious and its development partner BioInvent International signed a partnership agreement with the Neuroblastoma and Medulloblastoma Translational Research Center (NMTRC) to accelerate the clinical development of TB-403 for the treatment of medulloblastoma in the US. The NMTRC is a non-profit organization with the mission to bring forward new effective therapies against neuroblastoma and medulloblastoma. Headquartered at Helen DeVos Children's Hospital in Grand Rapids, MI, NMTRC is a network of 18 leading university hospitals and pediatric clinics in the US.

In May, a Phase I/IIa study was initiated with TB-403. The study, which is being conducted by NMTRC, aims to recruit 27 patients with Relapsed or Refractory Medulloblastoma.

JETREA Update

JETREA® Regulatory & Markets Access

ThromboGenics' first commercial drug JETREA® is now approved in 54 countries and reimbursed in over 20 countries.

Since its first introduction, around 25,000 patients have received a treatment with JETREA®.

The successful development of JETREA® demonstrates ThromboGenics´ pioneering role in developing pharmacological vitreolysis as a new drug class and the only pharmacological treatment option for symptomatic vitreomacular adhesion or vitreomacular traction.

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Advancing Science. Enhancing Vision.™

Ocriplasmin Research Findings Presented at EURETINA, EVER and AAO

Ocriplasmin research findings were presented at the European Society of Retina Specialists (EURETINA) in Copenhagen in September and in October at the European Association for Vision and Eye Research (EVER) 2016 in Nice and at the American Academy of Ophthalmology (AAO) meeting in Chicago.

15 ocriplasmin-related presentations, abstracts and posters were delivered at EURETINA. These covered preclinical research findings, real-world clinical data, and further characterization of results from different studies, including OASIS, and OVIID-I conducted in the US and Europe.

The data update confirmed the product's safety profile as described in the approved product label, with no new safety signals. Moreover, these new clinical studies and real-world data continued to confirm that appropriate patient selection leads to improved treatment outcomes.

Ocriplasmin clinical data were also presented during the **American Academy of Ophthalmology (AAO) Meeting** which took place from October 15 to 18 in Chicago as well as the EVER meeting which took from the 5th to 8th October in Nice.

Furthermore, the full report of the 2 year follow up of the **OASIS study** evaluating JETREA® (ocriplasmin) for the treatment of Symptomatic VMA/VMT and Macular Hole (n=220) has been published in Ophthalmology, Journal of the American Academy of Ophthalmology.

OASIS was designed to provide long term efficacy and safety data for JETREA® in a randomized, double-masked study in patients being treated for symptomatic vitreomacular adhesion (sVMA). The study included 24 months follow up data which is the longest period patients have been monitored post treatment with this novel medicine.

In its overall conclusion, Ophthalmology confirms 'the OASIS data demonstrates the long-term efficacy and safety of ocriplasmin, providing improved resolution of symptomatic VMA compared with previous phase 3 trials, with no additional safety signals identified'.

The article '*Results of the 2-Year Ocriplasmin for Treatment for Symptomatic Vitreomacular Adhesion Including Macular Hole (OASIS) Randomized Trial'* can be reviewed online here.

OASIS data, and its conclusions, have also been shared through presentations and publications during the recent AAO meeting in Chicago, *(Oct 15 -18, 2016).*

New 'Already-Diluted' Formulation of JETREA®

In June, ThromboGenics announced that the Office of Biotechnology Products of the U.S. Food and Drug Administration (FDA) had approved a new *already-diluted* formulation of JETREA® (ocriplasmin).

This new formulation of JETREA® offers the additional benefit of eliminating the current preparatory dilution steps prior to injection.

ThromboGenics Inc., which is commercializing JETREA® in the US, plans to launch the already-diluted formulation of JETREA® in the first half of 2017.

Financial review

At the end of September 2016, ThromboGenics had €88.2 million in cash and investments, compared to €91.5 million at the end of June 2016 and €101.4 at the end of December 2015.

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About ThromboGenics

ThromboGenics is a biopharmaceutical company focused on developing innovative treatments for diabetic eye disease.

The company's attractive pipeline of disease modifying drug candidates is targeting the key segments of the diabetic eye disease market. ThromboGenics is conducting the CIRCLE study, a Phase II clinical trial to assess THR-409 (ocriplasmin) as a potential treatment to prevent the patients with non-proliferative diabetic retinopathy progress to proliferative diabetic retinopathy. THR-317, a PIGF inhibitor being developed to treat diabetic macular edema, or as a combination therapy with anti-VEGF treatments, is expected to enter the clinic by year end 2016. In addition, 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, are in late stage pre-clinical development.

ThromboGenics pioneered a new drug category of pharmacological vitreolysis with JETREA® (ocriplasmin) which is now approved for the treatment of vitreomacular traction in 54 countries worldwide. ThromboGenics is commercializing JETREA® via its subsidiary ThromboGenics, Inc. in the US. Alcon (Novartis) commercializes JETREA® outside the United States.

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