THERANEXUS PUBLISHES ITS 2021 FULL-YEAR RESULTS AND ANNOUNCES A SHIFT OF ITS STRATEGIC FOCUS TO RARE NEUROLOGICAL DISEASES

Lyon, France – 25 April 2022 – 7:00 am CEST –Theranexus, a biopharmaceutical company innovating in the treatment of neurological diseases and pioneer in the development of drug candidates modulating the interaction between neurons and glial cells, today publishes its results for the year ending 31 December 2021 and announces its strategic shift to rare neurological diseases.

Theranexus Chairman and CEO, Franck Mouthon, c*omments: " As we are already engaged in rare neurological diseases since the choice of development in Batten disease, based on a detailed review of the situation and given the lack of industrial partner due largely to a shift of their strategic focus, we have decided to discontinue the THN102 program and to accelerate the shift towards these indications. Indeed, in order to optimize value creation for the company and its shareholders, we have chosen to rationalize our portfolio and focus our resources on treatments that directly affect the course of rare neurological diseases. The ambition of this strategic evolution is to conduct a pivotal trial in Batten disease, followed by a market launch, directly accessible for Theranexus. As part of this strategy, we focus our NeuroLead platform to accelerate the generation of new innovative therapy drug candidates acting on the biological mechanisms of rare neurological diseases. In line with this evolution, we have decided to discontinue the equity financing line put in place in January 2022. Please join us on 27 April for more detailed presentation of our strategic developments.*

Decision to discontinue THN102 for Excessive Daytime Sleepiness (EDS) in Parkinson's disease

Drawing on the positive Phase 2 results achieved by its drug candidate THN102 for Excessive Daytime Sleepiness in Parkinson's disease, the company resolutely explored all conceivable partnership options (global or regional licensing agreement, co-development, etc.). However, despite all efforts made during negotiations with several pharmaceutical companies, the discussions proved unsuccessful. Potential partners' recent strategic decision to move away from symptomatic treatments for Parkinson's disease, uncertainties regarding the commercial valuation of the product, and high development costs all prevented an agreement from being reached. The company decided to end its search for a partner and refrain from continuing the development process on its own due to the relative cost of completion, which is estimated at €100 million.

The company therefore decided to discontinue the THN102 program. Given the company's resources, the other two THN programs (THN101 and THN201), which are in an early clinical phase and involve broad indications that are highly capital-intensive, are also being discontinued. This decision will enable the company to focus its resources on its drug candidate under development for Batten disease and its NeuroLead platform, which aligns perfectly with its new strategic priorities.

2021 annual financial results and update on cash position on 31 March 2022

In €K	2021	2020
Turnover	-	-
Operating income	**25**	**315**
Other purchases and external charges	5,591	3,568
Wages and social security charges	2,689	2,422
Amortization and depreciation of fixed assets[1]	1,204	376
Other operating expenses	53	48
Operating expenses	**9,537**	**6,414**
Net operating income/(expenses)	**(9,512)**	**(6,099)**
Net financial income/(expense)	(396)	307
Income tax	1,758	994
Net income/(expense)	**(8,150)**	**(4,797)**

[1] Includes an impairment loss of €771,000 for the THN102, THN101 and THN201 patents and licenses and the platform patent

The 2021 full-year results were approved by the company's Board of Directors on 22 April 2022 and were the subject of a report by the company's Statutory Auditors.

In €K	31/03/2022*	31/12/2021
Cash available	**12,958**	**12,526**

** non-audited*

Other purchases and external charges rose in 2021 (€5,591,000) compared to 2020 (€3,568,000), since most of the regulatory work required to launch the clinical trial for Batten disease was completed in this financial year.

Wages and social security charges increased from €2,422,000 to €2,689,000, as the lower rate of social security charges was no longer applicable due to Innovative Young Company status coming to an end at the beginning of 2021.

The amortization and depreciation of fixed assets was affected by the decision to discontinue the THN102, THN101 and THN201 programs, resulting in an impairment loss of €771,000 (no residual value). However, this accounting expense had no impact on the company's cash position.

The variation in the net financial income/(expense) (€396,000 expense in 2021 compared to €307,000 income in 2020) is linked to the valuation of company shares held in connection with the liquidity agreement. This effect had no impact on the company's cash position.

Research Tax Credit due to be reimbursed in 2022 accounts for the large majority of income tax. This was higher in 2021 than in 2020 due to the increase in services, particularly in connection with research on Batten disease. Consequently, the net income/(expense) for 2021 was -€8,150,000.

As of 31 March 2022, Theranexus reported a cash position of €13 M giving it over 18 months' visibility. This cash position includes the payment of €1.7 M in advances and grants to fund NeuroLead and an €800,000 Bpifrance R&D Innovation Loan, which were both received in the first quarter of 2022.

This good financial visibility and the end of partnership negotiations for THN102 lead the company to interrupt the equity financing line set up with Iris Capital in January 2022.

The company's strategic shift to rare neurological diseases

Today, there are more than 5,000[1] rare neurological diseases affecting nearly 350 million[3] people throughout the world. 70%[2] of these conditions present during childhood and no treatment options are available for most of them. The global market for treating rare neurological diseases is set to double in size by 2027[3]. Products in these types of indications benefit from a regulatory framework encouraging development and commercial use, and faster market access.

Mathieu Charvériat, Deputy CEO and Chief Scientific Officer of Theranexus, states: *"We deploy our resources on the research and development of treatments for rare neurological diseases. Our choices are aimed at creating value simultaneously on the development and commercialization of our drug candidate Batten-1 in Batten disease and on our capacity to discover innovative therapy drugs. Our ambition is to commercialize the first drug that will radically change the lives of patients by 2026. Until then, we have chosen a development plan that will provide regular information on the efficacy of the drug candidate as early as 2023. Regarding our NeuroLead discovery platform, with the work carried out since the signature of the agreement with the BBDF Foundation, we now benefit from modelling capabilities for pathological neuron-glia interactions in rare neurological diseases, enabling us to make this strategic shift immediately, with the identification of a first asset within 18 months.*

- *An asset with high added value for Batten disease and an enhanced development plan*

In partnership with BBDF, Theranexus is continuing its juvenile Batten disease development program, for which it holds an exclusive, global license for development and commercial use. As a reminder, CLN3 juvenile Batten disease is a rare and fatal genetic nervous system disorder for which there is currently no approved treatment or cure.

BBDF identified the drug candidate BBDF-101, a combination of miglustat and trehalose. BBDF and Theranexus have new preclinical data demonstrating that miglustat exceeds expectations in terms of its efficacy. This combined with an improved understanding of the biology of Batten disease relating directly to miglustat's mechanism of action justifies its use in monotherapy. Based on that evidence, BBDF and Theranexus have decided to focus on miglustat in monotherapy and develop a proprietary oral liquid formulation of the product suitable for children, with this product being called Batten-1. The Phase 1/2 study of BBDF-101, launched in 2022, is being continued as part of the Batten-1 development process, as the trial is generating data on miglustat for Batten disease needed for initiating the pivotal Phase 2/3 trial of Batten-1. BBDF and Theranexus plan to initiate this pivotal pre-approval study in the first half of the 2023 financial year. The therapeutic goal of Batten-1 is to significantly slow Batten disease progression.

Based on their latest discussions with the Food and Drug Administration (FDA) in which it emerged that a single pivotal study with an added placebo arm could support the approval of Batten-1, BBDF and Theranexus will submit an adaptive design to the FDA. This study will be conducted with approximately 60 patients and will include an ongoing assessment of the effects of treatment compared to natural disease progression. Moreover, it will provide early and regular data on the efficacy of the Batten-1 drug candidate.

Simplified access to the market is in place, due to the existing partnership with the United States' leading patient association, BBDF, the opportunity of fast-track approval, the lack of competing projects at an advanced clinical stage, and also the high level of protection secured in Europe and the United States with a minimum of 7 years' exclusivity. Market potential is very high with estimated worldwide annual peak in sales of approximately €500 M.

[1] The Lancet Neurology, 2011
[2] Schule et al, Eur. J. Hum. Gen., 2021, Orphanet, NORD, EURORDIS
[3] *The Insight Partners, 2020*

- *NeuroLead, a platform for discovering advanced therapy medicinal products, which is now focused on rare diseases*

The company is currently focusing its discovery activities on the field of rare central nervous system disorders using its NeuroLead platform in collaboration with the Collège de France and the French Alternative Energies and Atomic Energy Commission (CEA). This unique platform equipped with the latest innovations in neuroscience is now harnessing its expertise on neuron-glia interactions and know-how in producing human pathological cellular models to identify new targets and develop advanced therapy medicinal product candidates for rare neurological diseases.

The company will present its new strategy in detail at 2.30 pm on 27 April at a webinar for investors: Click here: https://us02web.zoom.us/webinar/register/WN_TRliqfJtRpSbKKu0er5Iiw

About Theranexus

Theranexus is an innovative biopharmaceutical company that emerged from the French Alternative Energies and Atomic Energy Commission (CEA). It specializes in the treatment of central nervous system disorders and is a pioneer in the development of drug candidates targeting both neurons and glial cells.
Theranexus has a unique drug candidate of advanced therapy identification and characterization platform focused on rare neurological disorders and an initial drug candidate in clinical development for Batten disease.
Theranexus is listed on the Euronext Growth market in Paris (FR0013286259- ALTHX).

Next financial publication:

Tuesday 12 July 2022: Cash position as of 30 June 2022

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