Press Release

Pharnext Reports Full Year 2021 Financial Results and Recent Business Updates

Pharnext focuses its strategy and activities on its lead asset, PXT3003, in development for Charcot-Marie-Tooth Disease Type 1A ('CMT1A')

Company's vision is to become a leading biopharmaceutical company developing medicines for neurodegenerative diseases

Joshua Shafer appointed as Chairman of the Board

PARIS, France, April 25th, 2022, 10:00 pm CET – Pharnext SA (FR0011191287 - ALPHA) (the "Company"), an advanced late-clinical stage biopharmaceutical company developing novel therapeutics for neurodegenerative diseases with high unmet medical need, today announces its financial results for the fiscal year ended December 31st, 2021.

2021 KEY EVENTS

Clinical Development

Pharnext initiated the pivotal Phase III clinical study of PXT3003, the PREMIER trial. This study is an international, multicenter, randomized, double-blind, placebo-controlled clinical study, which will recruit around 350 patients with mild-to-moderate CMT1A in around 50 centers across the US, Canada, Europe, and Israel. Patients are treated for 15 months. The dose of PXT3003 tested in the PREMIER trial is equal to the high dose evaluated in the first Phase III study, the PLEO-CMT trial. The first patient was enrolled in the PREMIER trial in the US in March 2021 and in Europe in July 2021. Patient enrollment in the PREMIER trial is still on-going and on schedule for completion by the end of Q2 2022.

Pharnext is also conducting the open-label extension of the PLEO-CMT study, the PLEO-CMT-FU trial. In April 2021, the company announced data showing sustained benefits of a treatment with PXT3003 High Dose in patients with CMT1A after 54 months of total trial time (double blind and open-label extension studies: the PLEO-CMT and PLEO-CMT-FU trials). Treatment benefits were evaluated based on safety, tolerability, and efficacy (on the Overall Neuropathy Limitations Scale 'ONLS' evaluating the motor functional disability) parameters. Pharnext will continue to report annually on the safety and tolerability data, and the mean evolution of the ONLS score of patients enrolled in the PLEO-CMT-FU trial (around 130 patients as of today).

In October 2021, Pharnext announced the publication of the data from the first Phase III clinical study of PXT3003 in CMT1A, the PLEO-CMT trial, in the Orphanet Journal of Rare Diseases ('OJRD'). Based on their conclusion that the high-dose PXT3003 group demonstrated a statistically significant improvement in the primary endpoint, the ONLS Scale, compared to placebo, and a good safety profile, the authors state high-dose PXT3003 is considered a promising treatment option for patients with CMT1A. The full title of the PLEO-CMT trial publication in the OJRD is: "A double-blind, placebo-controlled, randomized trial of PXT3003 for the treatment of Charcot-Marie-Tooth type 1A"[1].

CMT Patients Associations

Over 2021, Pharnext has maintained its partnerships with CMT patient advocacy groups in the US and Europe. Notably, the Company has supported these groups in their disease awareness activities for the public on the occasion of International Rare Diseases Day in February 2021 and during the CMT Awareness months (in September 2021 in the US and October 2021 in Europe).

[1] https://ojrd.biomedcentral.com/articles/10.1186/s13023-021-02040-8

Senior Leadership Team Evolution

Pharnext strengthened its senior leadership team with three key appointments to support ongoing development of PXT3003 toward approval and commercialization:

- Raj Thota – Chief Manufacturing Officer and Head of CMC. Raj brings over 28 years of experience in pharmaceutical development, CMC filings, tech transfer, scale-up, and commercialization to Pharnext.
- Abhijit Pangu – Head of Regulatory Affairs. With over 20 years of pharmaceutical industry experience, Abhijit specializes in navigating drug development with global regulatory authorities.
- Xavier Paoli has been promoted to Chief Operating Officer. Xavier brings almost 20 years of experience in commercializing drugs in the biotech sector.

Financial transactions

On February 3, 2021, the Company raised gross proceeds of almost EUR 11 million through the issuance of (i) 1,754,386 new shares, each with a warrant attached (the "ABSAs") for a total amount of approximately EUR 6 million, and (ii) 5,473,685 bonds convertible into shares (the "OCA 0224s") for net proceeds of approximately EUR 5 million. As of today, 2,835,000 OCA 0224s were converted for a total amount of 974,429 ordinary shares, the balance was partially repaid in advance as of June 4, 2021, bringing the net proceeds to EUR 1,946,316 million.

On June 7, 2021, the Company announced the conclusion and implementation of a financing program for the issuance of bonds convertible into or exchangeable for new and/or existing shares (the "OCEANE-BSA") with Global Tech Opportunities 13. This program initially provided for the subscription by Global Tech Opportunities 13, at the request of the Company, and subject to the fulfillment of contractual and market conditions to be determined prior to each tranche drawdown, of a maximum of 35 OCEANE-BSA tranches over 36 months, representing a total of EUR 81 million. As of December 31, 2021, six tranches have been paid to the Company for a total gross amount of EUR 20.5 million and a net amount of EUR 16.83 million.

On December 22, 2021, the Company and Global Tech Opportunities 13, at the request of the Company, entered into an agreement providing for the termination of this financing at the end of May 2022 by limiting the number of OCEANE-BSA tranches to be drawn by the Company to five over the period from January to May 2022, corresponding to a gross nominal amount of EUR 15 million, subject to the fulfillment of contractual and market conditions to be determined prior to each tranche drawdown. This agreement was subject to (i) the signature of a "royalty agreement" (single digit percentage) on future net sales of PXT3003 during the life of the patent, which was signed in February 2022 and (ii) the approval of a specific authorization by a general meeting of the Company, which was held on March 21, 2022.

CONDENSED FINANCIAL INFORMATION

The main financial items are presented in the table below: they are taken from the financial statements prepared in accordance with IFRS standards and were approved by the Board of Directors at its meeting on April 25, 2022. The audit work has been carried out and the auditors' report on the certification is being issued.

Income Statement items (in € million) under IFRS at Dec. 31	2021	2020
Revenue from operations	0.085	0.040
Other Income	3.480	2.771
Administrative & Marketing costs	-6.808	-8.176
Research and Development costs	-19.614	-13.548
Operating result	-22.857	-18.914
Financial result	-7.761	-2.651
Net income	-30.618	-21.564

Cash flow statement items (in € million) under IFRS at Dec. 31	2021	2020
Cash flows generated from operating activities	-23.735	-16.082
Cash generated from investment activities	0.026	-0.061
Cash generated from financing activities	20.599	10.975
Net cash flow	-3.110	-5.168
Cash and cash equivalents	7.968	11.078

The full financial statements will be available by April 30, 2022, on the Pharnext website: www.pharnext.com

Other income is mainly composed of the Research Tax Credit (€3.2m for 2021 versus €2.6m in 2020), and grants.

Research and Development expenses have increased by €6m for 2021, mainly due to the Phase III clinical trial of PXT3003 (PREMIER trial). R&D expenses represent 74% of total operating expenses for the period. General and administrative expenses decreased by 22% and marketing expenses remained almost the same in 2021 compared to 2020.

Financial expenses amounted to €2.4m before IFRS restatement, mainly composed of interest on IPF bonds and interest on the OSEO DIPPAL grant. With regards to the IFRS treatment of financial instruments, they were not considered as equity instruments and consequently all fair value effects were classified as financial expenses for €6.2m. Also, because of this IFRS treatment, a positive financial result of EUR 1.0 million related to the fair value of the derivatives has been recorded in 2021. These IFRS financial entries are non-cash items.

The net loss for the period amounts to -€30.6m, compared with -€21.6m in 2020.

The number of employees is 40 at the end of the period, compared to 48 in 2020.

Borrowings in 2021 are comparable to those in 2020.

Balance sheet items (in € million) under IFRS at Dec 31	2021	2020
Cash and cash equivalents	**7.968**	**11.078**
Loans and borrowings	**21.914**	**20.947**

RECENT ACTIVITIES

On April 25, 2022, the Board of Directors reviewed and approved the proposal of focusing Pharnext's strategy and activities on its lead asset, PXT3003, in development for the treatment of CMT1A, with a vision to becoming a leading biopharmaceutical company developing medicines for neurodegenerative diseases. This new corporate focus has been linked to an internal reorganization leading to the departure of ten employees who were involved in the Pleotherapy R&D platform, and a reallocation of the Company's resources to further clinical development and new drug application submissions of PXT3003 in the U.S.A. and Europe.

On April 25, 2022, the Board of Directors of Pharnext appointed Joshua Schafer as Chairman of the Board, following his nomination as interim Chairman on March 28, 2022.

Pharnext has reinforced its commitment to rare diseases by supporting CMT patient organizations in awareness raising activities on February 28, 2022, on the occasion of the International Rare Disease Day.

Pharnext has strengthened its management team with the appointment of Valérie Worrall as Chief Financial Officer as of March 1, 2022.

An extraordinary general meeting of the Company was held on March 21, 2022, which granted a number of financial authorizations to the Company.

Over the period January 2022 up to this date, the Company received the payment of €12.5m gross, representing a net amount of €7.94m, corresponding to the payment of the 7th tranche and the drawings and payments of the 8th, 9th and 10th tranches of bonds convertible into shares, within the framework of the OCEANE-BSA issuance contract concluded with the Global Tech Opportunities 13 fund. In addition, a new delayed schedule for the drawdown of the remaining tranches has been agreed with Global Tech Opportunities 13 and provides that the payment of tranches 11 and 12 will run through June 20, 2022, for a total gross amount of €6m, representing a net amount in excess of €5.25m.

To date, the Company has cash of €7.66m, which gives it cash visibility for the financing of its operations over the next three months.

The Company is considering various financing options, including industrial collaborations, or licensing agreements with one or more manufacturers for its lead product candidate. The Company could also finance its future cash requirements through a combination of capital increases through public offerings or private placements, bank or bond financing, or other forms of financing.

About Pharnext

Pharnext is an advanced clinical-stage biopharmaceutical company developing novel therapeutics for neurodegenerative diseases that currently lack curative and/or disease-modifying treatments. Pharnext has two lead products in clinical development. PXT3003 completed an international Phase III trial with positive topline results for the treatment of Charcot-Marie-Tooth disease type 1A ('CMT1A') and benefits from orphan drug status in Europe and the United States. An international pivotal Phase III study of PXT3003 in CMT1A, the PREMIER trial, is currently ongoing. PXT864 has generated encouraging Phase II results in Alzheimer's disease and will be advanced through partnerships. Both of Pharnext's lead assets originated from the Pleotherapy R&D approach. Pharnext draws the attention of investors to the financial and other risk factors detailed in its financial reports. More information can be found at www.pharnext.com.
Pharnext is listed on the Euronext Growth Stock Exchange in Paris (ISIN code: FR0011191287).

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