

## **Pharnext Reports Financial Results for Year-End 2017**

**Paris, France, 11:55 p.m., April 12, 2018 (CEST) – Pharnext SA (FR00111911287 – ALPHA)**, a French biopharmaceutical company pioneering a new approach to developing innovative drug combinations based on Big Data genomics and Artificial Intelligence, today reported its financial and operational results for the year ended December 31, 2017.

### **2017 KEY EVENTS**

During 2017, Pharnext continued its international Phase 3 trial of PXT3003 for the treatment of Charcot-Marie-Tooth type 1A (CMT1A) disease. This pivotal 15-month trial is being conducted at 29 sites including 17 in Europe, 11 in the U.S. and one in Canada.

In September 2017, the independent Data Safety Monitoring Board (DSMB) completed its second pre-specified safety evaluation of PXT3003. Based on a review of safety data from all randomized patients, the DSMB recommended Pharnext continue the study as planned.

In November 2017, two additional interim analyses were conducted. These consisted of a blind variability analysis followed by a futility analysis. The results indicated that the PLEO-CMT study could continue according to the original plan, without having to increase the trial size.

In March 2017, Pharnext initiated an open-label extension study, which includes patients that completed the double-blind PLEO-CMT study. All patients received one of two doses of PXT3003 over an additional nine-month period. The initial results from this extension study are expected in the second quarter of 2019.

In May 2017, Pharnext signed a strategic agreement with Tasly Pharmaceutical (Shanghai: 600535), an organization ranked amongst China's top 10 pharmaceutical companies. This partnership included three components: a financial investment by Tasly in Pharnext comprised of €5 million in shares and €15 million in convertible bonds; the development of a new pipeline of synergistic combinations through a joint-venture; and the license of Pharnext's lead product PXT3003 to the joint-venture for commercialization in the Chinese market.

In March 2017, Pharnext signed a research and development (R&D) agreement with biotechnology company Galapagos NV (NASDAQ: GLPG). The primary aim of this agreement is to generate a pipeline of novel synergistic drug combinations in a broad set of indications.

## SELECTED FINANCIAL INFORMATION

The main elements of the year-end 2017 financials are set out in the table below: These are taken from the financial statements drawn up under IFRS, which were approved by the Board of Directors at its meeting on April 12, 2017. The audit procedures have been carried out and the auditors' report relating to the certification of the accounts is in the process of being issued. The full financial statements are available on the Company's website: [www.pharnext.com](http://www.pharnext.com).

| <i>In thousands of euros</i>                              | Selected financial information (under IFRS) |                 |
|---|---|-----------------|
|   | 2017  | 2016            |
| Operating revenues  | 3,324                                       | 4,436           |
| Research and development expenses                         | (15,529)                                    | (13,647)        |
| SG&A expenses   | (5,949)                                     | (4,177)         |
| <b>Operating income</b>                                   | <b>(18,153)</b>                             | <b>(13,389)</b> |
| <b>Financial income</b>                                   | <b>(1,922)</b>                              | <b>(4,058)</b>  |
| <b>Net income</b>   | <b>(20,075)</b>                             | <b>(17,447)</b> |
| Basic earnings per share (in €)                           | (2)   | (2.1)           |
| <b>Net cash flows used in operating activities</b>        | <b>(18,800)</b>                             | <b>(12,553)</b> |
| <b>Net cash flows used in financing activities</b>        | <b>15,361</b>                               | <b>26,902</b>   |
| <b>Net cash movement</b>                                  | <b>-4,215</b>                               | <b>13,581</b>   |
| <b>Cash and cash equivalents at the end of the period</b> | <b>12,454</b>                               | <b>16,670</b>   |

Operating revenues for 2017 were mainly generated by the Company's research tax credit (€3.9m in 2017) and subsidies.

The increase in research and development expenses were directly linked to the ramp-up of the PXT3003 Phase 3 clinical trial.

Financial income for 2017 was impacted by the recognition under financial expenses of the convertible bond issue in July for Tasly.

Net cash flows used in operating activities totalled €18.8m in 2017. The increase compared to the previous period was primarily the result of the Phase 3 roll-out.

Net cash flows used in financing activities in 2017 primarily comprised the €5m capital increase and the issue of €15m in convertible bonds for Tasly. In 2017, loan repayments and payment of interest charges and transaction costs on borrowing totalled €5.2m.

## ANTICIPATED UPCOMING MILESTONES

Pharnext will announce the results from PLEO-CMT, the Phase 3 clinical trial for its drug candidate PXT3003 in CMT1A, during the second half of 2018.

Beyond the 15-month PLEO-CMT study, patients will continue treatment in a 9-month follow-up extension study designed to assess the long-term safety of PXT3003. This follow-up extension study, which began in March 2017, will continue throughout the year. The initial results are expected in the second quarter of 2019.

Pharnext is planning to launch a Phase 3 paediatric clinical trial for PXT3003 in CMT1A in Europe and the United States.

In addition, after obtaining promising preliminary results in the Phase 2a trial for PXT864 in Alzheimer's disease and data in other neurodegenerative diseases, the Company has identified two additional strategic priorities for 2018:

- Prioritize and identify new orphan indications to launch internal Phase 2a studies;
- Continue to develop strategic collaborations in common indications.

## ABOUT PHARNEXT

Pharnext is an advanced clinical-stage biopharmaceutical company founded by renowned scientists and entrepreneurs including Professor Daniel Cohen, a pioneer in modern genomics: Pharnext has two lead products in clinical development: PXT3003 is currently in an international Phase 3 trial for the treatment of Charcot-Marie-Tooth disease type 1A and benefits from orphan drug status in Europe and the United States. The results of this trial are expected before the end of 2018. PXT864 has generated positive Phase 2 results in Alzheimer's disease. Pharnext is the pioneer of a new drug discovery paradigm based on Big Data genomics and Artificial Intelligence: PLEOTHERAPY™. The Company identifies and develops synergistic combinations of drugs. These PLEODRUG™ offer several key advantages: efficacy, safety and protected intellectual property. The Company is supported by a world-class scientific team.

Pharnext is listed on Euronext Alternext Stock Exchange in Paris (ISIN code: FR00111911287).

For more information, visit [www.pharnext.com](http://www.pharnext.com)

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