

## Pharnext Reports Year End 2016 Financial Results

PARIS, France, 6:00pm, April 26, 2017 (CEST) – Pharnext SA (FR00111911287 - ALPHA), a French biopharmaceutical company developing an advanced portfolio of products in the field of neurodegenerative diseases, today reported its financial and operational results for the year ended December 31, 2016.

### 2016 KEY EVENTS

Over the course of year, in line with the objectives announced at the time of its IPO, Pharnext rolled out the international Phase 3 trial of its lead clinical candidate PXT3003 for the treatment of Charcot-Marie-Tooth type 1A (CMT1A) disease: patient enrollment was completed in December 2016 as planned (323 patients in total across 3 arms). This pivotal 15-month Phase 3 trial is being conducted at 29 sites including 17 in Europe, 11 in the United States (U.S.) and one in Canada. The first pre-specified evaluation of PXT3003 safety data was conducted in November 2016 by the independent Data Safety Monitoring Board (DSMB). Based on this review, the DSMB recommended continuing the study as planned.

Regarding PXT864, its second lead drug in clinical development for the treatment of Alzheimer’s disease, Pharnext completed the results analysis of its Phase 2a study. The Company was invited to present these promising results during the annual CTAD (Clinical Trials on Alzheimer’s Disease) congress organized in San Diego in December 2016.

Pharnext’s listing on the Euronext Alternext stock exchange in July 2016, a key milestone for the year, enabled the Company to strengthen its equity and its cash by raising 30,9m€. The convertibles bonds held by historic shareholders were converted into equity.

### SELECTED FINANCIAL INFORMATION

The main elements of the year-end 2016 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 20, 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)

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Selected financial information (IFRS financial statements)		
<i>In kilo euros</i>	2016	2015
Operating Revenues	4 436	2 631
Research and Development expenses	(13 647)	(7 649)
SG&A expenses	(4 177)	(3 613)
<b>Operating Income</b>	<b>(13 389)</b>	<b>(8 631)</b>
<b>Net Financial Income</b>	<b>(4 058)</b>	<b>(2 364)</b>
<b>Earnings before tax</b>	<b>(17 447)</b>	<b>(10 994)</b>
Basic earnings per share (in €)	(2,1)	(1,7)
<b>Net cash flows used in operating activities</b>	<b>(12 553)</b>	<b>(9 173)</b>
<b>Net cash flows used in financing activities</b>	<b>26 902</b>	<b>5 257</b>
<b>Net cash movement</b>	<b>13 581</b>	<b>(4 148)</b>
<b>Cash and cash equivalents at the end of the period</b>	<b>16 670</b>	<b>3 089</b>

Operating revenues for the full year of 2016 was mainly generated by the Company's research tax credit (3,8m€ for the 2016 period) and subsidies.

The increase in Research and development expenses is directly linked to the deployment of the PXT3003 Phase 3 clinical trial.

The Financial income is affected, in 2016, by considering, at the level of financial costs, conversion premiums of convertible bond, subscribed in 2014.

Net cash flows used in operating activities were 12,6m€ in 2016: the increase compared to previous period resulted essentially from Phase 3 deployment.

Net cash flows used in financing activities consist mainly, in 2015, of a 5m€ venture loan, and in 2016, of a 2<sup>nd</sup> tranche of loan of 2.5m€, then of the net proceeds from the issuance of shares during the IPO (28.2m€). In 2016, loan reimbursements and payment of interest charges accounted for 3,5m€.

#### ANTICIPATED UPCOMING MILESTONES

Over the current financial year, Pharnext is continuing its Phase 3 clinical trial for its drug candidate PXT3003 in CMT1A. The last patient dosed is expected to complete this 15-month pivotal study in March 2018. In between, a futility analysis will be initiated in October 2017. Results of the futility analysis will be published as soon as they will become available.

Beyond the 15-month pivotal study, for which top-line results will be reported during the second quarter of 2018, patients will continue their 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.

In parallel to the above mentioned clinical studies, the Company also initiated new preclinical studies to complement the existing synergy data on PXT3003. At the end of the 15-month pivotal study, if positive, Pharnext plans to submit an application dossier for PXT3003 marketing authorization to the European regulatory authorities (EMA: European Medicine Agency) and U.S. authorities (FDA: Food and Drug Administration).

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In addition, after obtaining promising preliminary results in the Phase 2a trial for PXT864 in Alzheimer's disease, the Company will continue the design of the protocol of a Phase 2b study, which is expected to launch by the end of 2017.

Finally, following the signature of a research and development partnership early in March with Galapagos NV, on the creation of a new pipeline of novel synergistic drug combinations, Pharnext plans new agreements with other parties.

Thus, Pharnext is currently in advanced discussion on several international industrial strategic agreements. These agreements would contain business licenses, development of new pipelines and minority investments in equity with a premium on the current stock market price.

### 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 focuses on neurodegenerative diseases and 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. PXT864 has generated positive Phase 2 results in Alzheimer's disease. Pharnext is the pioneer of a new drug discovery paradigm: PLEOTHERAPY™. The Company identifies and develops synergic combinations of repositioned drugs at low dose. These PLEODRUG™ offer several key advantages: efficacy, safety and intellectual property including several product or composition of matter patents already granted. The Company is supported by a world-class scientific team.

The company 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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