Press release

First-Half 2017

- **Two strategic partnerships completed**
- **Major investment by Tasly Pharmaceutical**

- **Target confirmed: first marketing authorization of PXT3003 in the second half of 2019**

Paris, France, 5:45 pm, 19 October 2017 (CEST) – Pharnext SA (FR00111911287 - ALPHA), a biopharmaceutical company pioneering a new approach to the development of innovative drugs based on the combination and repositioning of known drugs, today announced its first-half 2017 financial results.

Daniel Cohen, M.D., Ph.D. Co-Founder and CEO said of activity for the first half of 2017: "*First-half activity was very dense; we implemented two prominent strategic partnerships with the biotech company Galapagos and the Tasly Group, one of the top ten pharmaceutical companies in China. Our flagship product, PXT3003 for the treatment of Charcot-Marie-Tooth disease type 1A, is nearing the end of Phase 3, which is slated for the second half of 2018. We confirm our target of taking the product to market by 2019.*"

A half-year marked by strategic agreements and the continued clinical trial for PXT3003 in Charcot-Marie-Tooth disease type 1A (CMT1A)

In March 2017, Pharnext signed its first R&D partnership with Galapagos NV for the creation of a new pipeline of pre-clinical combinations. This agreement relies on Pharnext's drug research and development platform: PLEOTHERAPY™. The working programme involves not only the therapeutic indications initially considered by Galapagos (inflammatory diseases), but also other indications that Pharnext can identify.

In May 2017, Pharnext also signed a major strategic agreement with Tasly Pharmaceutical, a group in the top 10 of listed Chinese pharmaceutical companies. This agreement is for a €20 million investment by Tasly in Pharnext and the creation of a Joint-Venture (JV) for research and development, 30% owned by Pharnext, to develop new combinations between chemical molecules and drugs produced by modernised Chinese traditional medicine. This partnership also includes a licensing agreement for the JV to market the drug candidate PXT3003 for CMT1A on the Chinese market.

During the first half of 2017, Pharnext continued the Phase 3 clinical trial of PXT3003, PLEODRUG™ candidate, for the treatment of CMT1A. Patient enrolment was completed in compliance with the established roadmap.

The date is set for year-end 2017: results of adaptive design and futility analysis in the Phase 3 clinical trial PLEO-CMT

The Phase 3 clinical development programme for PXT3003 continues. An important step is expected by the end of the year 2017 with the results of adaptive design and futility analysis.
The aim of the adaptive design analysis is to determine whether or not more patients should be added to the study. Futility analysis helps determine whether to continue or discontinue the trial, based on observed efficacy on the first patient sample (100/300).

High visibility at large international events

Along with ongoing R&D efforts, there was an increased presence at major international scientific meetings dedicated to orphan and neurodegenerative diseases, and to the repositioning of drugs:

- In late March 2017, Pharnext presented new synergy data in Vienna, Austria, at the 13th International Conference on Alzheimer's and Parkinson's diseases and related neurological disorders. In pre-clinical models of Alzheimer's disease, PXT864 enhanced the efficacy of standard therapeutic treatments.

- In late June 2017, the R&D PLEOTHERAPY™ platform was presented during the 6th Annual Conference on "Drug Repositioning, Repurposing and Rescue" in Chicago, United States. This presentation also gave Pharnext the opportunity to provide more specific information on its business model and growth strategy for the coming years.

- In early July 2017 in Sitjes, Spain, a status update of the pivotal Phase 3 clinical trial for PXT3003 in CMT1A was presented during an oral presentation and on a poster at the 2017 Peripheral Nerve Society Meeting.

- In mid-July 2017 in London, United Kingdom, new synergy data on PXT864 in Alzheimer's disease were also presented at the Alzheimer's Association International Conference 2017.

- In early September 2017, status update of the pivotal Phase 3 trial of PXT3003 was also presented as a poster at the 2017 American Association of Neuromuscular & Electrodiagnostic Medicine (AANEM) in Phoenix, United States.

From now until the end of the year, Pharnext will participate in major international events where it will have the opportunity to spotlight the clinical development of PXT3003 and PXT864 to the global medical and scientific community. For instance, the company will give a presentation at the CMT Patient Summit of the Hereditary Neuropathy Foundation (HNF). This will be held in Boston, United States, on 3 November. The company will also take part in the CTAD (Clinical Trial on Alzheimer's Disease) conference in Boston, United States, from 1-4 November.

H1 2017 Financial Results

as €K [(1)] – IFRS at 30 June	H1 2017	H1 2016
Other income	**1,216**	1,993
Research & development expenses	**(7,610)**	(5,740)
Administrative costs	**(2,936)**	(1,927)
Operating income	**(9,330)**	(5,674)
Financial income	**(767)**	(2,295)
Net income	**(10,098)**	(7,969)
Net cash flows generated from (used in) operating activities	**(12,108)**	(3,692)
Net cash generated from (used in) investment activities	**(152)**	(296)
Net cash generated from (used in) financing activities	**1,740**	5,907
Change in cash and cash equivalents	**(10,521)**	1,919
Cash and cash equivalents	**6,149**	5,008
Pro forma cash after Tasly investments	**26,149**	

(1) *The H1 2017 financial statements were approved by the Board of Directors at their meeting on 19 October 2017. They were subject to a limited review by the Statutory Auditors. The half-year financial report is available on the Company's website:* https://www.pharnext.com/en/investors/

As the company is not yet seeing any revenue, most other deferred revenue comes from the research tax credit (Research Tax Credit for H1 2017 of €K 1,956, and the French Tax Credit for Competitiveness and Employment (CICE) to the amount of €K 10,2).

The increase in R&D spending is related to the development of Phase 3 trials on Charcot-Marie-Tooth disease (CMT 1A).

The operating loss at 30 June 2017 was -€9.3 million, compared to -€5.6 million one year earlier. The decline in financial income is largely due to lower interest charges. After accounting for these elements, net income came to -€10.1 million compared with -€7.9 million at 30 June 2016.

Cash flow requirements generated by the activity amounted to €12.1 million in the first half of 2017. Cash flows from investments came to -€K 152. Resources generated by financing activities stood at €1.7 million.

At 30 June 2017, the company's share capital was negative at -€6.4 million. Cash assets amounted to €6.1 million before Tasly operations.

Post-closing, the agreements signed with Tasly Pharmaceutical had several financial impacts in July, with the income from a restricted capital increase, a €5 million cash contribution (€4.9 million issue premium) and a €15 million convertible bond issuance.

Restated for these two transactions, pro forma cash assets at the end of June 2017 after the Tasly Group's investment amount to €26.1 million.

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 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 synergistic combinations of repositioned drugs at optimal reduced doses. These PLEODRUG™ have many significant benefits: effective and innocuous, their solid intellectual property includes several product patents already delivered. The Company is supported by a world-class scientific team.

The company Pharnext is listed on Euronext Growth Stock Exchange in Paris (ISIN code: FR00111911287).
For more information, visit our website: *www.pharnext.com*

UPCOMING EVENTS

Salon Actionaria, Paris: 23 & 24 November 2017
Publication of 2017 annual results: 27 April 2018

CONTACTS:

Financial Communications (France and Europe)
Actifin
Stéphane Ruiz
sruiz@actifin.fr
+33 (0)1 56 88 11 11

Investor Relations (U.S.)
Stern Investor Relations, Inc.
Matthew Shinseki
matthew@sternir.com
+1 212-362-1200

DISCLAIMER

This press release contains certain forward-looking statements concerning Pharnext and its business. Such forward-looking statements are based on assumptions that Pharnext considers to be reasonable. However, there can be no assurance that such forward-looking statements will be verified, which statements are subject to numerous risks, including the risks set forth in the Document de référence registration document filed with the Autorité des marchés financiers (AMF- French Financial Market Authority) on July 28, 2016 under n°R.16-069 and to the development of economic conditions, financial markets and the markets in which Pharnext operates. The forward-looking statements contained in this press release are also subject to risks not yet known to Pharnext or not currently considered material by Pharnext. The occurrence of all or part of such risks could cause actual results, financial conditions, performance or achievements of Pharnext to be materially different from such forward-looking statements. This press release and the information that it contains do not constitute an offer to sell or subscribe for, or a solicitation of an offer to purchase or subscribe for, Pharnext

shares in any country. The communication of this press release in certain countries may constitute a violation of local laws and regulations. Any recipient of this press release must inform oneself of any such local restrictions and comply therewith.