CORPORATE NEWS

EARNINGS

PAION AG PUBLISHES GROUP QUARTERLY STATEMENT FOR THE FIRST NINE MONTHS OF 2016

- Positive remimazolam data in pivotal U.S. Phase III study for procedural sedation during colonoscopy
- Investment and license agreement with Cosmo Pharmaceuticals for development and commercialization of remimazolam in the U.S.
- Strong cash position of EUR 35.9 million as of 30 September 2016
- Conference call (in English) today at 2:00 pm CET (1:00 pm GMT/8:00 am ET)

Aachen (Germany), 09 November 2016 – The specialty pharmaceutical company PAION AG (ISIN DE000A0B65S3; Frankfurt Stock Exchange, Prime Standard: PA8) today reports its consolidated financial results according to International Financial Reporting Standards (IFRS) for the first nine months of 2016.

"*The positive Phase III data from the U.S. colonoscopy study as well as the Cosmo deal have significantly de-risked PAION's profile and contributed to a very successful year to date. Through the agreements with Cosmo, our financial position has improved significantly and the data from the first U.S. Phase III study in procedural sedation have been received very positively from both proceduralists and anesthesiologists not only in the U.S. The presentation by Prof. Rex at the ACG meeting in the U.S. was very well attended and has resonated very positively also beyond the conference. Based on the feedback that we received, the most intriguing part of the remimazolam data were the significant improvements in onset and recovery times as well as restoration of cognitive function and fewer adverse events when compared to the control groups*", Dr. Wolfgang Söhngen, CEO of PAION AG, commented and added: "*So far, we are still in the process of evaluating our options for Japan. It also took longer than expected for the U.S., were we now have a good partner and a clear path forward."*

Important events in the period covered

In the first nine months of 2016, PAION focused on advancing its Phase III program for the development of **remimazolam** in procedural sedation in the U.S.

U.S.

In April 2016, patient recruitment in the first study of PAION's pivotal U.S. Phase III program in patients undergoing procedural sedation was completed,

and in June 2016, PAION announced that the study's primary efficacy endpoint had been met.

The Phase III trial enrolled 461 patients at 13 U.S. sites and was designed to evaluate the efficacy and safety of remimazolam compared to placebo (with midazolam rescue) in patients undergoing proceduralist-administered sedation for colonoscopy.

The primary outcome measure was a composite endpoint defined as: no need for rescue medication, completion of the procedure and no more than 5 top-up doses within any 15-minute window. The primary endpoint was reached in 91.3% of the patients in the remimazolam arm and 1.7% in the placebo (including midazolam rescue) arm.

Further data on the clinical results of remimazolam's U.S. Phase III colonoscopy trial were presented at the 2016 American College of Gastroenterology (ACG) Annual Scientific Meeting in Las Vegas on 18 October 2016 (after the period covered).

Important secondary endpoints in the remimazolam arm showed a median time from start of medication to start of procedure of 4.0 minutes (placebo 19.5 minutes) and a mean time from end of procedure to return to full alertness of 7.2 minutes (placebo 21.3 minutes). Additionally, time to "back to normal" as reported by patients on remimazolam was 331 minutes (placebo 572 minutes). There were no treatment-emergent serious adverse events in the trial. Hypotension was 44.3% with remimazolam and 47.5% with placebo and accounted for most of the adverse events in all study arms. Hypoxia occurred in 1.0% of patients given remimazolam, 3.4% in the placebo arm. On the Hopkins Verbal Learning Test administered five minutes after reaching the fully alert status, the total raw score, delayed recall, memory retention, and recognition discrimination scores were all better with remimazolam compared to placebo. Patient satisfaction was similar in all arms of the study.

In addition to the detailed analysis of the primary and secondary endpoints of the Phase III trial (comparison to placebo), Prof. Rex also presented data for the open label midazolam arm. These results will not be part of the label claims. They will however serve as valuable data to plan future studies and perform pharmacoeconomic modelling. Midazolam patients showed a median time from start of medication to start of procedure of 19.0 minutes and a mean time from end of procedure to return to full alertness of 15.7 minutes. Midazolam patients took 553 minutes to be back to normal. Hypotension was 67.3% with midazolam and hypoxia occurred in 1.0% of patients given midazolam.

In addition to the above study, the U.S. Phase III program includes a second confirmatory, prospective, double-blind placebo-controlled, randomized, multi-center open label vs. midazolam trial in 420 patients undergoing bronchoscopies. Patient recruitment was initially moderate. However, continuous measures to accelerate patient recruitment, such as improvements in the feasibility of the study protocol, opening additional study centers and intensified support of existing study centers have increased the recruitment rate and PAION therefore confirms the previously communicated timeline for completion of patient recruitment in the second quarter of 2017. Over half of the 420 patients have already been recruited to the study.

A smaller safety trial in high-risk patients (double blind vs. placebo and open label vs. midazolam) undergoing colonoscopies for which patient recruitment is expected to be completed in 2016, and four Phase I trials to further support remimazolam's safety profile complement the U.S. development program.

In June 2016, PAION entered into an investment agreement and license agreement for remimazolam U.S. rights with Cosmo. Under the terms of the license agreement, Cosmo received an exclusive license for the development and commercialization of remimazolam in the U.S. and will bear all future costs related to the market authorization as well as sales and distribution of remimazolam. PAION remains responsible for and bears the costs associated with the completion of the ongoing U.S. clinical development program in procedural sedation. PAION received an upfront payment in the amount of EUR 10 million in July 2016 and is eligible for further payments of up to EUR 42.5 million depending on the achievement of certain regulatory milestones in the U.S. PAION is also eligible to receive tiered royalties upon commercialization ranging from 20% to 25% of net sales (which may be adjusted under certain conditions but not to below 15%).

Under the terms of the investment agreement, Cosmo has committed to invest EUR 10 million in PAION shares, which are subject to a 12-month lock-up period upon purchase. In June 2016, Cosmo already invested EUR 9.6 million in a private placement; the remaining EUR 0.4 million will be invested at a later date.

EU

In February 2016, PAION discontinued a confirmatory EU Phase III study with remimazolam in patients undergoing major cardiac surgery. Due to the complex study design, the trial faced recruitment challenges. PAION is currently evaluating how to resume clinical development in the EU.

Japan

In January 2016, PAION held a pre-NDA meeting with the Japanese Pharmaceuticals and Medical Devices Agency (PMDA). The PMDA stated that they considered the non-clinical and clinical data package for remimazolam to be complete for filing for the indication "Induction and maintenance of general anesthesia" in Japan. The PMDA previously had confirmed that both the raw materials produced by PAION in Europe, as well as the finished formulation of remimazolam, fulfilled the requirements for filing in Japan.

China

In September 2016, Yichang Humanwell, PAION's Chinese remimazolam licensing partner, announced that their first Phase I study started in October 2015 completed patient recruitment. In addition, Yichang Humanwell has started a second Phase I study with continuous infusion in order to prepare a study in general anesthesia. A Phase II study in procedural sedation in China is in preparation.

Yichang Humanwell had initially planned to develop remimazolam for general anesthesia for the Chinese market but decided to develop remimazolam in procedural sedation as well in September 2016.

Partner activities in other regions

All license partners continuously perform activities to support future filings in their respective territories with a focus on regulatory interactions.

Results of operations, financial position and net assets

Revenues in the first nine months of 2016 amounted to KEUR 2,230 compared to KEUR 44 in the prior-year period and mainly resulted from the remimazolam license agreement with Cosmo that PAION entered into in June 2016.

Research and development expenses amounted to KEUR 16,431 in the first nine months of 2016 and decreased by KEUR 4,476 compared to the prior-year period. The decrease is mainly attributable to the progress of the Phase I trials of the U.S. development program. Expenses for Phase III development were incurred on a comparable level to the prior-year period as higher costs for the U.S. program in procedural sedation were offset by lower costs for the EU program in general anesthesia.

General administrative and selling expenses decreased by KEUR 346 to KEUR 4,190 in the first nine months of 2016. General administrative expenses increased by KEUR 504 to KEUR 3,171 while selling expenses decreased by KEUR 850 to KEUR 1,019. The increase in general administrative expenses primarily results from the preparation of potential capital measures that were ultimately not conducted in light of the agreements entered into with Cosmo. Selling expenses mainly comprise costs related to the initiation and preparation of license agreements in the reporting period, while in the first nine months of 2015, selling expenses primarily included market research as well as pre-marketing and market access activities.

Other income (expenses) amounted to KEUR -1,116 (prior-year period: KEUR 684) and includes foreign exchange losses in the amount of KEUR 1,337, while in the prior-year period, other income (expenses) comprised foreign exchange gains amounting to KEUR 786.

Income taxes in the first nine months of 2016 amounted to KEUR 3,430 (prior-year period: KEUR 4,301) and relate to tax claims for reimbursement of parts of the research and development costs from the British tax authorities.

The **net loss** for the first nine months of 2016 amounted to KEUR 16,061 compared to a net loss of KEUR 20,391 in the prior-year period. This means a decrease of the net loss in the amount of KEUR 4,330 compared to the prior-year period. The change is mainly attributable to higher revenues and lower research and development expenses on the one hand and higher other expenses than in the prior-year period on the other hand.

Cash and cash equivalents amounted to KEUR 35,906 as of 30 September 2016, an increase of KEUR 3,226 compared to 31 December 2015.

The increase of cash and cash equivalents primarily stems from **cash flows from operating activities** of KEUR -5,799 and **cash flows from financing activities** of KEUR 9,199. Cash flows from operating activities mainly result from the net loss, the tax credit payment from British tax authorities received in May 2016 adjusted for the current tax credit claim towards the British tax authorities which has not had a cash effect yet, as well as the portion of the upfront payment from Cosmo received in July 2016 that has not yet been recognized as revenues. Cash flows from financing activities primarily result from the private placement conducted with Cosmo in June 2016.

Risks and Opportunities

Material risks and opportunities relating to future development are presented in detail in the group management report for fiscal year 2015. Due to the positive results from the U.S. Phase III colonoscopy trial and the agreements entered into with Cosmo, the risk situation has substantially improved. Other than that, material risks and opportunities have not changed significantly in the first nine months of 2016.

Outlook

PAION confirms its outlook for the current fiscal year announced on 10 August 2016 with the publication of the half-year results for 2016. PAION's major goals for the remainder of 2016 are the continuation of the ongoing Phase III development program with remimazolam in the U.S. and the implementation of the cooperation with Cosmo, including the necessary know-how transfer. In addition, PAION continues to work on production development for remimazolam, in particular the validation of commercial-scale production, as well as on the preparation of filing for market approval in the U.S. PAION expects its other regional partners to continue their remimazolam development activities.

In the U.S., PAION is allocating significant resources to achieve the planned completion of the Phase III program, especially on patient enrollment in the Phase III bronchoscopy study. The timing to submit for U.S. market approval following successful completion of the clinical development program is subject to ongoing discussions with Cosmo, who is responsible for all approval activities in the U.S.

Subject to completing ongoing U.S. development activities and the associated know-how transfer to Cosmo, PAION intends to resume development activities for remimazolam in the EU. In this context, the next step is to re-evaluate the studies already conducted in general anesthesia. Along these lines, in close collaboration with key opinion leaders in the EU, PAION is analyzing the design and feasibility of a new development program as a basis for discussion with the European Medicines Agency (EMA).

Following the positive pre-NDA meeting with the Japanese authority in the beginning of 2016, in which the details of a market approval for remimazolam were outlined, PAION is continuing partnering discussions with potential licensees. Alternatively, PAION is also evaluating the option of filing for market approval itself with the aim to partner during or after that process. The preparation of a Japanese dossier would require additional funding. Such a dossier could serve as a reference dossier for approval in certain other markets.

Financial Outlook

PAION expects revenues of approximately EUR 4 million in 2016 resulting primarily from the upfront payment of EUR 10 million received from Cosmo in July 2016. The remaining approximately EUR 6 million of the upfront payment are expected to be recognized as revenues in 2017. This financial outlook assumes that no further license agreements are entered into during 2016.

Due to the ongoing clinical development of remimazolam, PAION expects research and development expenses to be between EUR 24 million and EUR 27 million for 2016, depending on the progress of development. Income from tax credits on parts of the research and development expenses from

British tax authorities is expected to be between EUR 4 million and EUR 4.5 million. General, administrative and selling expenses are expected to amount to approximately EUR 5.5 million. Net loss is expected to be between EUR 21.5 million and EUR 24 million, a decrease compared to the previous year (2015: EUR 28.2 million).

This outlook assumes that development activities for remimazolam in the U.S. will progress as expected. If not, a significant portion of these costs would instead be expected to occur during 2017. Expense forecasts are also based on the current status of discussions with the U.S. Food & Drug Administration (FDA). Costs could be higher than planned and lead to a delay in approval should the FDA impose additional requirements for filing for market approval.

Based on current plans, PAION believes that cash and cash equivalents of EUR 35.9 million as of 30 September 2016 enable PAION to complete ongoing Phase III development, including the preparation for filing of remimazolam in the indication procedural sedation in the U.S. Thereafter, PAION expects to receive further payments from Cosmo subject to the achievement of certain regulatory milestones in the U.S., and, once remimazolam is approved, royalties on net sales. Should development, filing and approval go according to plan, PAION will not need additional funding to bring remimazolam to the U.S. market. Regarding the EU, PAION is currently evaluating how to resume the clinical development of remimazolam. For further development in the EU or own filing activities in Japan, additional funding would be required. The amount will be specified after the ongoing evaluation of a potential new development program has been finalized.

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Key consolidated financial figures, IFRS (not audited)

(all figures in EUR thousand unless noted otherwise)	Q3 2016	Q3 2015	Q1-Q3 2016	Q1-Q3 2015
Revenues	2,032	5	2,230	44
Research and development expenses	-4,276	-8,927	-16,431	-20,907
General administrative and selling expenses	-940	-1,812	-4,190	-4,536
Net result for the period	-2,858	-9,051	-16,061	-20,391
Earnings per share in EUR for the period (basic)	-0.05	-0.18	-0.31	-0.40
Earnings per share in EUR for the period (diluted)	-0.05	-0.18	-0.31	-0.40

	Q1-Q3 2016	Q1-Q3 2015
Cash flows from operating activities	-5,799	-18,427
Cash flows from investing activities	-149	-26
Cash flows from financing activities	9,199	22
Change in cash and cash equivalents	3,226	-18,373
Average number of employees in the Group	38	28

	30-09-2016	31-12-2015
Intangible assets	2,681	3,362
Cash and cash equivalents	35,906	32,680
Equity	28,887	35,562
Non-current liabilities	35	6
Current liabilities	13,411	7,900
Total assets	42,333	43,468

Conference call and webcast

In addition to the publication of results, the Management Board of PAION AG will host a public conference call (conducted in English) on 09 November 2016 at 2 p.m. CET (1 p.m. GMT, 8 a.m. ET) to present the financial results for the first nine months of 2016, highlight key events and provide a pipeline and strategy update and financial outlook.

To access the English call starting at 2 p.m., participants may dial from

- Germany +49 (0) 69 7104 45598,
- UK +44 (0) 20 3003 2666 and
- U.S. +1 212 999 6659
- Other countries: please use the UK number

When prompted, you will be asked to give the password “PAION”. The conference call will be supplemented by a webcast presentation which can be accessed during the call under the following link: https://paion-events.webex.com/paion-events/j.php?MTID=m2fd560deda136e65c6520720c12d978c.

About PAION

PAION AG is a publicly listed specialty pharmaceutical company developing and aiming to commercialize innovative drugs to be used in out-patient and hospital-based sedation, anesthesia and critical care services. PAION’s lead compound is remimazolam, an intravenous, ultra-short-acting and controllable

benzodiazepine sedative/anesthetic drug candidate. Currently, remimazolam is in active Phase III clinical development for use in procedural sedation in the U.S., where PAION is focusing all its business and financial resources on successfully completing its ongoing clinical development program in procedural sedation. Outside the U.S., PAION has so far focused on the development of remimazolam in the indication general anesthesia. Development of remimazolam in the indication intensive care unit (ICU) sedation is also part of the longer term life-cycle plan for remimazolam.

PAION is headquartered in Aachen (Germany) with further sites in Cambridge (United Kingdom) and New Jersey (USA).

PAION's vision is to become an acknowledged “PAIONeer” in sedation and anesthesia.

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